Advisory Panel To Better Understand and Make Recommendations Regarding the Implications of Genome-editing Technology for the Citizens of the State

Wednesday, August 17, 2022 (9:00 a.m. to 4:00 p.m.)

Live transcription / closed captioning generated by Zoom

09:05:50 Good morning, everyone, and welcome to the first several meetings on what is an advisory panel to better understand and make recommendations regarding the implications of genome editing technology for the citizens of Maine

09:06:06 We will get to introduction shortly, but just by way of some basic things, I want to thank the panel members for taking the time to be here and be in person, both the ones who are in person and the ones who are remote thank

09:06:18 you for deciding that this is an important enough topic that it's worth a significant consideration, and your time during main summer we are very grateful for that commitment.

09:06:32 We're grateful for the folks who are in the room. and for those of you who are watching on Youtube as we try to work our way through some of these things for those of you who have not been here But before.

09:06:44 The bathrooms are down the hall to the left mans is the first women's is the second on the other side of the corridor.

09:06:52 We have a tradition of trying to keep food away from everything at our desks.

09:07:00 But rick is fine to keep hydrated and we try to build in some breaks to our morning, so that folks don't get too rooted. It's also part of the tradition.

09:07:12 Is, if you feel like you need to stand up you can certainly stand behind your chair.

09:07:16 That's considered fair there's a little room back in there you can go into that.

09:07:20 So away, and then there's almost the rest of the buildings. this is pretty quiet today, so I just mentioned those things for you.

09:07:28 There's a cafeteria downstairs moving at hot drinks and some food.

09:07:34 We'll talk about that later generally we'd like to hold questions until after the presenter is done to make sure they have a chance to get through all of their intended material, and the only time question makes particular sense to

09:07:50 be answered right away is if it's someone uses an abbreviation, or an acronym that you don't understand, which may well happen frequently in this work.

09:08:02 But we'll save some time for questions and if we can't get it to all of them the chair in our and I are committed with the analyst to going over any questions that people have want to have wanted to have raised and

09:08:14 try and get answers for the next meeting or cigarette which ones need more discussion.

09:08:18 So we'll work on that if you have any questions about all of this kind of stuff representative zager the Honorable Dr.

09:08:27 Hymensen, who's sitting over here? and representative Arada?

09:08:34 How are you doing? and the honorable tina riley are also available, since they've hung out in this building for a while before.

09:08:42 I wanna make sure I publicly thank the folks in it for all the work that went into getting this up and running.

09:08:49 You may notice that there is a scrolling translation on the zoom link.

09:08:55 We'll go over how to get to that or set that up.

09:08:58 If you haven't already i'll turn it over to the staff in a minute.

09:09:03 But it's required a lot of work in a building that was not designed for this.

09:09:08 I haven't heard lots of echoes so I assume that that means everybody in the room here turned off the Sound on their computer, and we'll rely on the mics in front of us with will light up red when you're

09:09:21 using it when you're being when you've been recognized So with that, I would like to go to Representative Zaker. did you have some things that you wanted to contribute to at this point, or should we

09:09:37 go to introductions. thank you eric clock tonight. just for the sake of brevity. I I also want to extend my deep appreciation to everyone who's involved participating and and staffing this

09:09:50 certainly want to call out janet Staco and Rachel Olson for their ability to turn concepts and ideas into actual meetings and and tangible things.

09:10:02 We'll get more into details. After we do some introductions

09:10:07 So that's that's all I wanted to say at this point very good, thank you.

09:10:11 Alright. so we have a number of panel members here? Let's start with dana Vateman, do you wanna turn on your mic Is your introduce yourself with who you?

09:10:22 Are where you came from, and what brings you to this work?

09:10:26 Sure. Thank you. thank you for having me so again. I'm Dana wearing Bateman.

09:10:34 I am the co-founder of the Personal Genetics Education Project, which is a organization based at Harvard Medical School.

09:10:42 That is dedicated to engaging people of all walks of life around emerging issues in genetics.

09:10:51 Whether it be, you know, medical issues ancestry as well as sort of broader questions around.

09:10:58 You know how we want to use genetics in our personal lives, and our most sort of like, you know, intimate private lives as well as sort of the public.

09:11:07 Questions that genetics brings to us criminal justice system environmental questions.

09:11:14 I'm the former education director. and now i'm a freelancer.

09:11:19 And I live here in Brunswick, Maine, and I am happy to be here.

09:11:26 And my background is actually in the history of science so I am not like a lab person.

09:11:34 I'm glad there are many lab and medical people here with us today.

09:11:39 Thank you. Thank you. We try to make it for some of the first representation.

09:11:41 The Honorable Tina Riley

09:11:46 You turn your mic on she's out of practice Good morning.

09:11:53 Sorry about that. i'm happy to be here this morning it's definitely an honor.

09:11:59 I served 2 terms in the house on the energy committee and stayed as far away from the hhs committee as I could.

09:12:10 I have a son that you're going to hear a little bit more about a little later on today who is affected by a single gene gene disorder, dishon muscular dystrophy and

09:12:20 i'm here to share that experience just my lived experience with you, Thank you.

09:12:28 Thank Thank you. I didn't mention the introduction that one of our objectives with this work is to surface as many.

09:12:35 It problems as quickly as problem as possible. so we're we're on our way with that.

09:12:40 So, thank you. we'll find out more I understand Abby Honeywell is available.

09:12:48 Can you? turn on your wife and join us? Yeah. Hi: Thank you.

09:12:57 I am from Maine i'm actually living with a single gene disorder.

09:13:02 I'm currently attending the usm studying public health.

09:13:06 So. I have quite an interest in this topic in general, but also with my lived experience.

09:13:10 I think that it's gonna definitely be interesting and i'm excited to be here today.

09:13:17 Well, thank you for taking time to join this work. I see Markus Houston

09:13:28 Hi! everyone, Thank you. i'm excited to be here my name is Marcus Houston.

09:13:32 I live in Portland, Maine, originally from Monmouth, Maine,

09:13:38 And I am the person under 30 serving on this this p advisory panel.

09:13:45 I don't have a background working directly with genetics, or in this field.

09:13:49 But have worked in the past with Lots of folks who live with single gene disorders and have seen the impact that it can have on their lives.

09:14:00 So looking forward to working through all this with you, and Hope hopefully coming up with some good solutions, so we can move forward as a state.

09:14:08 Thank you. Thank you. Lowest Lowry, You care to introduce yourself.

09:14:13 Hi there! I'm Lois Lowry I'm speaking to you from my summer home in Richmond Maine.

09:14:21 Sorry I can't be with you in person I i'm told that a guster is a place you can't get there from here. I could have had I had I set out early this morning.

09:14:35 But thank goodness for technology that enables this to happen i'm, i'm here as the person representing.

09:14:43 I guess I saw on the list you needed a writer or poet. I'm. i'm hardly a poet, but I am a writer.

09:14:52 I have now, I think, 50 books published, 2 more coming out in the year to come.

09:14:59 None of which dale explicitly with science.

09:15:03 But I I do grapple with questions of the future, and my primary audience is young people.

09:15:12 Those who will be our future. So this is of interest and concern to me.

09:15:21 And assuming I can make all this technology work i'm looking forward part of it.

09:15:25 Thank you. Well, thank you for being here. we also have.

09:15:30 Did I see Dwayne Toma I see on not here today.

09:15:39 Okay, Thank you, Frank. Chessa

09:15:45 Good morning, everyone, and thanks for inviting me to the Panel I'm Francesa.

09:15:51 I'm. director of Clinical ethics at Main Medical Center, and an assistant professor of medicine at Tufts University,

09:15:58 I work at main medical so Well, so I'm, the bioethicist member of the panel.

09:16:04 At main medical Center, my my work and bioethics fans everything from direct, patient care and the ethics consult service to work in research, compliance and and doing a little bit of research myself, and then a good deal

09:16:21 of education of our young physicians in training.

09:16:28 So so very happy to be here just by way of background.

09:16:32 I have a a doctor to PHD. in ethical theory from Georgetown University, where I concentrated in medical ethics and bioethics.

09:16:44 So very happy to be here. Well, thank you again for taking the time. Representative Arada.

09:16:52 Good morning. My name's amy Erada and I represent House District 65, which includes New Gloucester and part of Poland, and in a previous phase of life.

09:17:03 I had them. I received a Master's degree in Genetics at the University of California.

09:17:07 Davis, and so i'm looking forward to learning more about how genetics has progressed in the years since I've stopped working in the laboratory.

09:17:17 Thank you. Welcome representative Patricia Hymensen.

09:17:25 Representative Patti Hymens and I live in York, and I represent a house district 4 which is parts of York.

09:17:31 Well, Sanford and Oliver Gonquit and Representative Rata and I sit next to each other in in the Appropriations and Financial Affairs Committee, and I had no idea that you head into that experience so you learn

09:17:46 something new every day, So in our citizens in our citizens Legislature. I am also a physician.

09:17:52 I'm. a neurologist I was in private practice in the seacoast area for 26 years in patient and outpatient, and in that capacity I also served as the chairman of the Ethics Committee Medical

09:18:05 Ethics Committee at Portsmouth Regional Hospital for 10 years.

09:18:11 I also did some genetics research between collagen medical school in a lab that was inserting genes into bacteria fages.

09:18:26 So that was very early research in the 19 or eighties.

09:18:31 So i'm been watching this genetic story go for many years.

09:18:38 Neurologies filled with hope and for curing some very lethal disorders using genetic techniques.

09:18:52 But i'm also aware of the ethical challenges so I'm really happy to be on this panel, and, as I've been saying, put my nerd on Senator Marianne Moore

09:19:07 Good morning. I'm Marianne Moore and I represent Senate District 6, which is all of Washington County, as well as Goalsboro, Winter Harbor and Sullivan in Hancock County.

09:19:17 As well, and I was, I currently serve on the health and human services committee.

09:19:20 It's a part of the this. The major discussions that we had regarding the bill and the bills.

09:19:28 There was multiple bills that were involved with that, and was very pleased with what came the outcome out of the bill and the formation of the advisory panel.

09:19:39 We had, like, I said, some long, hairy discussions.

09:19:41 So this is a great compromise that came out thanks to Senator Claxton as well.

09:19:46 So i'm excited to be here to participate in this important panel.

09:19:50 Thank you, Senator BaldacchiThank you.

09:19:57 Senator Cla. sounds good to hear from you and see everyone i'm excited to be here as well.

09:20:02 I am Joe Valdashi. I represent the city of Bangor and the town of Herman in San Francisco.

09:20:08 9. I have been in private practice as an attorney for 31 years.

09:20:14 Do not have a genetics degree I have a lot Doctor law degree, and so i'm your the committee's lawyer in case that's needed.

09:20:21 But yeah, so and I'm take There's a large healthcare sector in Bangor Northern Light.

09:20:30 Same shows this large healthcare sector in terms of employment and investment.

09:20:35 So this, obviously we'll have implications here and all over. but I also take the more of the lay person's perspective and and hoping that we can. these challenges, particularly regarding bioethics.

09:20:50 I look forward to learning a lot, and I appreciate it.

09:20:53 Raise time. Thank you. Representatives say here, Thank you so much.

09:20:59 Senator Paxton. it's great to see everyone I'm. Sam Zager I'm.

09:21:06 Honored to represent a house district 41 in which is part of Portland.

09:21:08 It's off peninsula in Portland in my other life.

09:21:12 I am a family physician, and I, in the course of training and caring for patients of all different backgrounds and experiences, have become increasingly aware of the role that genetics has has played.

09:21:32 And how this is in any emerging, and actually very much in front of us.

09:21:37 Frontier. in our society and that humanity is facing and and i'm this which led me to introduce the legislation that led to this committee being formed.

09:21:49 So. thank you everyone for participating i'm looking forward to fruitful, lively, and broad discussion, and i'm Ned Claxton.

09:21:59 I serve Senate District 20, which consists of Auburn mine.

09:22:03 It. mechanic Falls, Poland, New Gloucester,

09:22:09 And I was cheer the Health and Human Services Committee when this bill first came up for discussion.

09:22:15 Dr. Representative Zeger has undersold how hard he pushed to make this happen. and I need to credit him for that.

09:22:26 And as Senator Moore alluded to it was a very animated conversation. The committee focused a while to find some common ground to get to what this turned out to be so I'm a family physician by

09:22:41 training. I am a legislator by accident and i'm I count every day that I get to do this work as a good day, and and one that i'm fortunate to be able to enjoy I didn't

09:22:54 see on the screen. but apparently have missed Justice Mohammed, or see you on the screen somewhere, that I'm not seeing there you are, Welcome, sir.

09:23:07 Welcome, sir. good morning, my name is rick mulher and i'm a spear court justice currently sitting in your county spear court in Alfred.

09:23:16 I've been a spirit court justice for a year and a half, and before that I was a district court judge for 13 years, and before that a trial lawyer in Portland for 22 years. I'm.

09:23:24 Here as the Chief Justice designee she's just Stanford's testing.

09:23:30 He is member of the Committee I was a biology Major, as an undergraduate. so I've always been interested in science, and I actually knew what Crispr was before getting this time.

09:23:39 I read about it in the technology. So i'm looking forward to being a member of this committee and and learning more about this this important area.

09:23:47 Thank you. Thank you for your participation. The other people we wanna make sure we introduce are the 2 folks who have made this whole committee come together, and I reference Rachel Olson, you wanna introduce

09:24:01 yourself. Hi, everyone! Good morning. My name is Rachel Olson.

09:24:05 I am an analyst in the office policy. and legal analysis. and I am one of your 2 staff individuals who's gonna help the panel do its work, and the other is Janet Stoco.

09:24:18 Hello, I'm janet stoko i'm. also a legislative analyst, and I currently serve for the Vla. Committee, which I refer to as the sin committee. You know that elections are

09:24:30 veterans in legal affairs, Right? Yes, the veterans are not simple.

09:24:36 And Rachel serves on the Ida Committee. Just so.

09:24:41 You know where we serve Yeah. that's the idea Committee idea and

09:24:58 We are very excited. We are also ready to nerd out video representative.

09:25:05 They've already started. So these these 2 folks who just introduced themselves have been incredibly important to the evolution of the agenda.

09:25:15 The invite you list the process and helping us focus where we might get to before we get started.

09:25:25 I and I turn it over to them I do want to reassure folks that we will not be considering all 10,000 single gene just in detail.

09:25:36 We're trying to keep focused on the implications for Maine.

09:25:42 In the next 5 to 10 years, maybe trying to look over the horizon a little bit.

09:25:46 And what do we want to do with the advent of significant gene editing as it emerges in society to help the best help and serve the people in made?

09:25:57 So that'll be our Continue focus so at this point i'll turn it over to

09:26:04 The crew, the exemplary support staff. What was reward?

09:26:10 Stellar stellar this is still our crew. Okay? Good.

09:26:18 Morning I'm gonna start out with some technical items.

09:26:24 I'll start with technology first, because it's not on my to do list.

09:26:27 So i'll start with that first so I won't forget about it.

09:26:30 I think everyone's done it but if you need to rename yourself on zoom.

09:26:35 Please don't forget to do so we are using a transcription service that Senator Claxton mentioned at the beginning.

09:26:43 I believe we are the guinea pigs for doing this.

09:26:47 You can see it on the screen in the room, and hopefully everyone on the live stream is also able to view it.

09:26:52 And if you would like to have it on your personal device, the way that you would do that is by finding the live transcription symbol at the bottom of your zoom screen and clicking on the carrot and turning on the

09:27:07 subtitles so minor on, so it says hide.

09:27:11 But if they were not on it would say, show, Okay,

09:27:18 If you are in the room and you are using a personal computer device, and everyone's doing an excellent job so far.

09:27:24 But please remember to turn off the microphone and the audio on your personal device, and if you are in the room, you need to speak, using the microphone that's in the room piece remember to turn it both on and off when you were

09:27:37 done. and I think that's my quick overview for technology.

09:27:42 We're all doing really really well this morning so far so we're getting those glitches out of the way.

09:27:48 Also mention and start with one of the handouts in your packets.

09:27:52 You did receive from Janet this this week a number of materials, and if you are in the room, or if you join us at a later meeting. we have packets for you as panelists.

09:28:07 That contain a number of important items. one of those is the resolve which I will go back to in just a moment that formed the panel.

09:28:16 But i'll go over a couple of other things that you'll find inside

09:28:20 You have a copy of today's agenda you have a copy of the slides that will accompany Miss wearing Vateman's presentation this morning as well as the credits to go along with those slides and you can

09:28:32 find all of those on the committee's website as Well, Janet.

09:28:38 We'll talk about that shortly. We do have a handout regarding Covid.

09:28:40 19 that we'd ask you to review and if you have any questions to let us know.

09:28:48 You also find the letter from the chairs printed on our letter head. that was emailed to you and is now formally in your package.

09:29:00 We have our membership list You have a copy. of the it's a printout of the background materials that are available on the website. These are This is just a printout So you need to go to the website.

09:29:13 For the active links. It also contains links to the enabling Legislation, Ld. 1,771, as well as a previous piece of legislation, related Ld.

09:29:25 1,601, and links to testimony, and then finally,

09:29:29 We have a printed copy of one of the background articles from the New England Journal of Medicine, and although we've emailed you this, it would behoove us to mention that we have permission only to have made enough copies and

09:29:43 to distribute to to the panelists that we please ask You do not copy or distribute this any further or more widely, as we do not have copyright permission, for that there are no questions about your packet.

09:30:00 I will direct you all to the resolve and my job this morning before I do. You want to go before I do it? Okay, My job before I hand it off to Janet is to talk about your duties as panelists

09:30:16 So, as panelists, your duties are to study the implications of genome editing technology.

09:30:26 The legislative, administrative or other steps that the State should take to capitalize on the potential of genome editing technology and avoid the hazards of genome editing technology.

09:30:37 And to do your work, you, as the panel shall solicit the testimony, advise, or participation of persons having a very wide range of expertise.

09:30:48 Many of you on the panel and the panelists that we will invite both today and in later meetings we'll cover many of these, including ethics.

09:30:56 The clinical medicine, caring for children, Clinical medicine, caring for adults, public health, bioscience, research, environmental protection, forestry, agriculture, or aquaculture, Fishing State economics, tourism.

09:31:12 business or commerce, military or security affairs, the University of main System, or the main community college system.

09:31:22 Individuals living with a single cell, a single gene disorder.

09:31:24 Excuse me, or a parent or guardian of a person living with such a single gene, disorder, hospital, or hospice, chaplaincy and history of race, ethnicity, or eugenics.

09:31:36 So quite a long list of expertise and i've all heard that we're ready to nerd out so I don't think we'll be too too concerned to tackle that and i'll remind you

09:31:46 that. we are getting a a early start, and thank you all for joining us this early in August.

09:31:54 We have until November second to complete our work.

09:31:58 Technically it reads: The report is due November second.

09:32:01 I know many of you are familiar with this process, and know the official report will by no means be ready.

09:32:07 November, the second in a perfect world. Everyone will have seen a draft before we finish our work, although much of that will probably come by email near the end That November second date date is interpreted really to me that our work is the

09:32:21 panel. Your work as the panel needs to be completed by that date, although there is the ability to ask for an extension.

09:32:27 Should we need one? I love extensions? hey, miss?

09:32:37 Yeah. and the report is going to include findings and recommendations that the panel has come to, including suggested legislation.

09:32:49 That will be sent to the joint standing committee of the Legislature having jurisdiction over health and human services, and that committee can report out legislation on the first regular session.

09:33:00 Of the 1 30 first. so your job is to come up with findings and recommendations to send to the next legislature, and they may or may not choose to take action.

09:33:12 Thank you, Rachel again i'm janet one thing I do want to say at the beginning.

09:33:19 Because this is the interim, and we do not take vacation any other time of the year.

09:33:23 We are variously out at different times so If you ever need to email us whether you're listening as a member of the public right now, or you're on the panel or you're a presenter please always try and copy both of

09:33:35 us, because that will help you. make sure you get the answer as quickly as possible, in case one of us does happen to be out.

09:33:45 I did want to show you our website of background material or of all of the materials actually, just so that you see. and also so that we can test this screen sharing before we make dataatements share share her screen can make sure

09:33:59 it's working today, or we'll share it for you don't worry.

09:34:03 So this is the advisory panel's web page if you're watching on the public.

09:34:07 You've already figured out how to get to the stream so you probably know this, but it is kind of difficult to find study web pages.

09:34:17 If you've ever looked you actually have to go up to the top of the legislature's web page here to legislative offices, and you go through oprah that's our acronym for office policy and

09:34:30 legal analysis where both Rachel and I work, and if you go through that website you can find the studies.

09:34:35 But if you somehow, miraculously, sign up for the interested parties list, we have sent you the link to the website, and we will send it to you many more times.

09:34:43 So don't worry. You will see it so here, we have we do have a copy of the Resolve, so for folks who are watching from the public.

09:34:52 You can read through the resolve that Rachel just summarized for the advisory panel members.

09:34:57 We will have materials for each of the meeting dates and you'll see.

09:35:00 We know what 3 of the meeting dates are those may change if it turns out panel members aren't available.

09:35:06 We haven't heard that yet, but if that happens we may change them.

09:35:08 But these are the dates. we know of we're currently figuring out the fourth date, and you will see that appear on here when we know what it is.

09:35:17 Anytime you click on one of those dates, you will get to the materials for that meeting.

09:35:22 So we have the agenda posted here, We have Miss bateman's presentation posted here.

09:35:28 And then she went through, and very meticulously followed all kinds of copyright laws, and gave credits for all of her images and links, even to some of them.

09:35:35 So that is also posted here as well. right now.

09:35:41 It says how to live stream the video So people who are watching already know this, and that's where the link is.

09:35:48 But just to reassure folks, we will put a link to the actual recorded live stream here later.

09:35:54 Once we have it because it is a little bit difficult to find the archives. We have switched away from using the Youtube platform to the Legislature's own platform, and until we all get used to binding all archive meetings we're

09:36:06 just going to give you the direct link here we don't have it yet, because the meeting is not over.

09:36:11 Then, at the bottom of this page you'll see rachel's name in my name, and if you click on those you'll get our email address.

09:36:18 So if you ever forget, and you say, oh, gosh Dan, it told me to email both of them, I can't remember their email addresses.

09:36:23 You can find them right here, you will find a list of all of your own names for advisory panel members.

09:36:29 Here as well. So, if you remember Oh, I met this lovely person, and I really want to talk to them.

09:36:35 I forget their name, and what they're affiliation was you can just click on that and find out.

09:36:41 And then we also have all the background materials so this is the page that's printed in your folder.

09:36:45 But if you luck, all of the links are to all of the materials here, the one just for people who are watching from members of the public, Rachel did mention that in the folder of the members is an actual article

09:36:59 It's a opinion piece from the New England journal of medicine that one, If you click on our link, it will bring you to the New England Journal of Medicine Homepage, where you can sign up to access it?

09:37:11 I think they do give you 2 free articles so it's Not that it's going to cost you money, but just for advisory panel members, we did go and get permission to print it out.

09:37:20 For them, but it's not that the public can't access it you just have to go through the journal's web page just to be respectful of their copyrights.

09:37:29 And then the final thing I wanted to talk to you about and then you won't have to listen to me anymore.

09:37:36 Is just the freedom of access act, and how it applies. because not everyone here is a legislator who's had this beaten into their head many times by opa analysts.

09:37:46 So the freedom of Access Act is a main law. You hear Foia a lot at the Federal level.

09:37:52 We were actually foa, because it's a state government public proceedings.

09:37:57 So this is a public proceeding that's why we are going to great lengths to make sure the public can attend in person.

09:38:02 There are some people here today which makes me really happy we haven't had that in a long time.

09:38:08 The public can also watch the live stream we are recording it. we're going to try and see if we can get a recording of the transcript.

09:38:17 This is new. So if we can, we'll post that as well, so people who need to see that can see that as well to go along with the video, it also means, however, that every time you engage in discussions as a panel that is

09:38:31 a public proceeding, and this can be very important, because you may go back home today.

09:38:36 We talked about nerding out. This is a fascinating topic.

09:38:40 We talk about this with the chairs all the time, and you may just be really eager to start discussing it.

09:38:44 And now you'll have everybody's email addresses because Rachel and I have emailed you. but once you start doing that, you are having a public meeting in private because you're all emailing each other about the advisory

09:38:57 panel business. So just be aware of that i'm trying not to have substant discussions.

09:39:02 Obviously one person talking to another is not an advisory panel meeting

09:39:06 It's clear in a legislative committee context. Once you have 3 people involved.

09:39:12 You have a public proceeding going on so just kind of keep that rule of thumb in mind.

09:39:16 It's not directly applicable to an advisory panel but it's a good rule of thumb You start having a larger discussion.

09:39:21 You are having a public meeting and be aware that the public is not part of that email chain.

09:39:27 This actually will become of greater importance later on, when we send you the report draft that we will get your comments on. we will instruct you, and we will remind you at that point to just write back to Rachel and myself, and we

09:39:40 will take those comments, compile them and discuss them with the chairs, because if people start riding back and forth to each other, that is definitely a public meeting, because you're all discussing what should be the content of the report and again the

09:39:52 public's not invited to that it's not that you're being the fairies.

09:39:56 It's just something, you might forget about so we'll just remind you of it.

09:40:01 Another thing to be aware of, because it is a public proceeding.

09:40:05 If someone wants to submit, a request they may submit a request, a full request, and get copies of the emails that you're sending, you're not sending anything to terrible So it's not a problem.

09:40:14 But just so that you're aware that could happen because this is a public meeting.

09:40:19 So I think that covered all of my things. So now you can get to the real heart of the matter.

09:40:27 It also gives me a chance to acknowledge again how dependence the chairs have been.

09:40:30 My these 2 we would get us organized and how valuable they've been in this conversation, so thank you again.

09:40:37 I see we've been joined by julia finn and i'm wondering if she's here in her capacity as a designee from the governors are arena she could speak for herself if you want

09:40:52 but she is the legislative Liaison for the judicial branch, and I think she's here, because just as small hern is part of this.

09:41:00 Then Justice Stanford designated him. but if you wanna have a talk.

09:41:04 Obviously I do welcome. Yes, that's correct i'm with the judicial branch, and i'm really just here as an observer. I actually thought i'd be in some sort of invisible waiting room.

09:41:17 So surprised to see my name pop up and I can go and just stream through the legislative website.

09:41:23 If that is preferable. it, doesn't matter to us we just wanted to make sure you felt welcome.

09:41:27 And thank you for being here. Alright, thank you. All right.

09:41:32 So that gets us to the next set of remarks. propose a purpose and expectation, general orientation.

09:41:39 Do you want to go with that? Thank you so much Senator Claxton?

09:41:43 I i'm gonna ask invite people to think of a time in your life when you needed to be there for somebody maybe somebody that you know and love.

09:41:54 Maybe it's a complete stranger that'd say you just happen to be on the street with and a time when you you were asked to to make a difference to to weigh in and do something I have a a a friend of mine in andrew

09:42:06 who told me about a time in his life when this happened he happens to be a doctor, and was waiting on an airplane on the tarmac waiting to take off. and if anybody here has ever been on an airplane waiting on

09:42:19 a tarmac. You know that that can be a lengthy process.

09:42:23 It. The boredom was setting in and and then there was a little commotion, some seats away, and then a tense voice from a flood attendant over the intercom i'm, asking if there's a medical professional

09:42:34 on board, Andrew responded, was scattered to a man unfortunately clearly suffering a heart attack.

09:42:45 There was another physician on board that, and both of them asked for any equipment, any medical.

09:42:52 The kit that they had on board. they were not able to quickly get that to the gate, and so this was a a difficult clinical situation.

09:43:02 They tried to do. Cpr. which was really difficult in

09:43:07 The cramped space of of an aircraft.

09:43:12 Andrew was told me that he was. he was wondering.

09:43:17 Is is this family is this man's family gonna watch his demise here, is you know what's gonna happen here.

09:43:25 They were doing the best they could with with all the training that they had and experience that they had.

09:43:30 But what they really needed was a tool. They really needed something called an Aed automatic.

09:43:34 It's external defibrillator this is a laptop size device.

09:43:39 Many people here may know a bit but if you don't it's a laptop size device that can analyze a person's rhythm.

09:43:46 See if they're having a so-called shockable rhythm and deliver that shock fortunately Airlines stock.

09:43:56 This on pretty much every larger aircraft they had one on board. but Andrew also knew that, unlike in the movies that usually does not work, but it's all they had is all they is the last hope they had so they they hooked.

09:44:12 Up. the This gentleman initiated the sequence. You analyze the rhythm found to be shockable.

09:44:19 They shocked him, and he re He recounts his pulse, recovered his started breathing again.

09:44:26 He was literally resurrected from the moment of death.

09:44:31 Andrew, I am At this point the The pilots had brought the aircraft back to the gate.

09:44:37 They offloaded the the patient to paramedics who were waiting. and Andrew, when all the excitement ended, started to shake uncontrollably, and he said that everyone was congratulating and thanking him and this other

09:44:51 fellow, and he said, Sam, they were calling us heroes we weren't heroes.

09:44:57 That machine. Those things are amazing, he said. You know, regardless of how much training and experience they had on in inpatient floor in an idealized setting, where you can do a lot of things, and you have a whole team of folks it was the

09:45:10 right tool that somebody had developed and had actually taken centuries to develop.

09:45:15 When you think back to somebody at some point. Maybe it was Ben Franklin.

09:45:20 According to American folklore, somebody said wait there's lightning or the static electricity.

09:45:22 What is that? Could we harness that? Is there some good that can come of that eventually?

09:45:28 That was harness. used to like rooms like this and

09:45:34 And and then eventually, a couple centuries later, somebody developed a Aed to save a life, an incredible use of technology.

09:45:42 But in considering that technology, we also must recall that this very same phenomenon can be used to end a life and has been has done many times.

09:45:52 The electric chair is in other States, never in Maine, but has been used many times since.

09:45:57 1,890 to execute convicted criminals.

09:46:03 We now know with genetic investigation and forensics, that unfortunately, despite the best of interests and a lot of professionalism, we get it wrong.

09:46:16 We had we condemned the wrong people where we don't condemn.

09:46:18 We don't meet our justice. any of egalitarian fashion.

09:46:24 So technologies can be phenomenal, but they are introduced onto a landscape.

09:46:32 Human landscape that has history, ethics, law, economics, many factors on that landscape, and there's, of course, populating that landscape are human beings.

09:46:46 Our neighbors, our loved ones, ourselves. Everyone in this State whose lives are going to be affected by technologies

09:46:57 With each new technology, there's. a set? of questions that comes up. The basic question is, okay, and how that we can do this harness, electricity or edit genes.

09:47:10 Whatever it is, the question is, what do we do with it don't we do with it?

09:47:16 Who gets to decide In what manner do we improve on this?

09:47:24 How do we invest our finite resources to just to pursue that hopes of questions?

09:47:31 We are not going to answer all these questions that is not what we that's not within the scope dance.

09:47:38 We are, we are starting broad discussion. This is the first body of its kind.

09:47:46 To our knowledge in the world, really to to to take this approach, to look at all the ways that we can think of, to get the right questions on the table, to get the right people in the room, and to do this in an open in

09:48:02 an open fashion. as Senator Moore mentioned.

09:48:07 This body is the result of a lot of forthright discussion.

09:48:14 Good faith, compromise, and and and a devotion, to do what's right to to try to figure this out as we as a society, as we as a state, move into this new era.

09:48:26 And this is a new era in human history. So without making it 2 2 grand I i'd like to just get back to some nuts and bolts as we try to extract all the good all the benefit from

09:48:43 this technology, or in, you know, in the future and Also, if you're clear of the potential pitfalls.

09:48:49 So a few nuts and bolts as has already been alluded to. and mentioned by Janet and Rachel.

09:48:56 Today is the first of 4 plan meetings. other dates in September.

09:49:00 And welcome a letter in the website. we've got today.

09:49:07 Is where Dana wait a minute there.

09:49:11 Bateman wearing is going to be giving us a a starting point for technical understanding of what this technology is this afternoon.

09:49:19 We're going to hear testimony from What we're calling group A, which is titled Gene Editing in Health and Bioscience.

09:49:26 It's it's a good place to start and health and medicine is is where much of gene editing has been applied to date, but just like computers started off just in The space.

09:49:37 Program, and then it quickly eventually proliferated everything in society.

09:49:41 September seventh we're gonna have experts in group B which will be gene editing in the natural world, exploring regarding environment, agriculture, alcohol for far forestry fishing group c

09:49:54 also that on that day will be gene editing in humanities which will include history, ethics, and faith perspectives.

09:50:02 The 20 first of september we'll have group d which will be gene editing in systems and institutions.

09:50:07 So we'll look at things like a state economy business legal system education and defense.

09:50:14 National security, which may, of course, plays a role in in other ways.

09:50:18 So each subject matter expert will have 8 to 10 min, will then have approximately 10 min or so to ask questions.

09:50:24 Everyone on the panel, so can certainly ask questions we have to be mindful of time.

09:50:31 We don't have infinite time by any means and each on each day, and as increasingly as we proceed through the days of this panel on the sessions will be formulating and discussing formulating recommendations that

09:50:43 will eventually be in the report which will all help refine at any point, feel free to verbally or in writing share with with staff or chairs.

09:50:58 What other information you would find useful. As we consider these questions, and and consider what other questions to ask.

09:51:10 A lot of the other. These other things have already been discussed.

09:51:16 I do want to thank the Hhs Committee that deliberated over this.

09:51:21 The legislature for passing it governor for signing this bill and the citizen funders who funded this bill. it's for us to be here today.

09:51:34 With that I think i'll i'll end it Senator, Have I missed anything?

09:51:40 No, just to recognize just to recognize that the funder was part of a nonprofit donation.

09:51:47 There was no agenda that's that's thank you thank you very much.

09:51:53 Sounds good. Alright, I'll turn it back to you so So now you have an even better sense of why we're here, and how how much work Sam Representative Zeker is done to get us here?

09:52:05 So with that in mind, and and looking at the clock it's unheard of.

09:52:12 But we're ahead of time and then legislative time.

09:52:19 So at this point scheduled let's make sure before we do that, does anybody have any questions at all about details?

09:52:27 Rudiments. I will mention that you can get to the chat function on the since i'm in charge of the Monday, you can get to the chat function if you are logged in but we ask that you only use

09:52:40 it, or procedural stuff, and not for content since that isn't captured.

09:52:47 So that would better be done on Mike or by email.

09:52:51 Okay. So how about if we take our 15 min break now, and we'll seed the extra 1520 min to our presenter.

09:53:08 If she would like to do that, that would also leave us some extra time or questions.

09:53:13 So with that in mind we'll take a 15 min break and be back at 10.

09:53:24 Oh, 8!

10:11:22 Back to order i'm gonna get out to grabble i'm, threatening them with a gavel

10:11:42 Representative Hymensen

10:11:49 I was threatening. I was threatening with the gavel

10:11:59 Okay, So we're back on Mike for the back live hit

10:12:05 Yeah, there's all better thank you we're a little later than intended.

10:12:11 But people were already starting to nerd out so in our attempts to try and make this a greatual process of increasing understanding.

10:12:23 We can now go to a more formal opportunity to understand some of the issues involved, and to that for that we turn to dinner wearing Bateman, and We'll at a request which is fine will she prefer

10:12:43 to take questions as she goes along. as an homage to her teaching background.

10:12:50 So. I defer to you and to your presentation.

10:12:53 Thank you for me and here to do it. thank you.

10:12:58 I appreciate it so. to your point I am a teacher by my background. so I would be happy to sort of entertain questions, comments, clarifications, as I go, and I have got a set of slides to that

10:13:18 i'm gonna use as a a sort of a guide for some of our conversation today.

10:13:24 I also have a short video that I am optimistic i'm going to be able to successfully share before you get to that.

10:13:31 Can I just point out that we'll you can use your electronic raise hand if you're not in the room, or you can do the wave hand in front of the camera routine.

10:13:41 And we'll be trying to watch the room too it gets a little complicated sometimes, so we'll try and keep an eye open in the chamber in the room here.

10:13:51 We'll have people put their hand up and now, we get seen.

10:13:54 But if you're not here feel free to attract our attention any way that works, thank you.

10:14:00 So I am going to start by sharing my screen

10:14:18 Okay.

10:14:37 Okay.

10:14:38 Okay. So again, Part of the part of where I come from is the Personal Genetics Education Project based at Harvard Medical School.

10:14:49 It's housed in the laboratory of Dr. ting Wu. who is a Traceophila geneticist or a fruit fly.

10:14:55 Genesis which you'd be surprised how much as humans and fruit flies have in common lots of our Dna.

10:15:03 In fact. we, there are sort of analogous genes in in fruit flies, and so

10:15:09 But in addition to that, that work in the lab, one of

10:15:14 Her interest, was looking at these questions. of you know all the work that they're doing in, you know, a genetics department like Harvard Medical School.

10:15:22 What's you know that's it's heading out into the world, and she felt an obligation as a research scientist to participate in build some infrastructure to have public conversations about these topics and I

10:15:37 should note that I I appreciated whoever shared that editorial by Dean Daley. George Daley at Harvard Medical School.

10:15:47 I should say he's the dean of the medical school where I had been employed, and he he you know some of our funding was through his discretion.

10:16:01 So that's a disclosure for me I thought today we could talk a little bit about like where things were at in genetics, both in terms of what we are able to like learn about our Dna, and Then this Crispr

10:16:13 is a real shift in thinking that it's not we aren't just like finding out what are the genes we have in our bodies.

10:16:22 What are they doing? How do they work it's this it's this new idea that we can now change them right?

10:16:30 So. many of us might be familiar with genetic testing.

10:16:35 Some of us have maybe received a 23, and me or a similar sort of kit.

10:16:41 That looks at both ancestry and health Those are now, you know there's many different companies, some specialize in

10:16:51 So certain communities, For example, there's some that are particularly focused on ancestry around African Americans.

10:17:00 People come to it for many different reasons. Some of the pioneers who, who, you know, were the first people to use these direct consumer genetic tests.

10:17:10 People who are adopted people who are separated from biological family for one reason or another.

10:17:18 People who were seeking, you know, some genetic analysis which has been hard to come by.

10:17:23 You know it's expensive. how do you you know many family doctors.

10:17:30 You know we're we're finding themselves in these situations where people were coming in with their 23 and me results, saying, Can you?

10:17:37 What do you think? right? and you know those were not clinical grade reports?

10:17:45 But you know there's a lot of confusion there certainly about them.

10:17:48 Those have now been around for 10 or 15 years. At the early days there was a lot of controversy.

10:17:55 Should we even sell these to the public directly, or the public ready for it?

10:18:00 It seems like overall. The public has been ready for it.

10:18:03 But there was a lot of controversy around making you know sort of democratizing that that information in in a way.

10:18:15 So i'm happy that we're starting with healthcare today. I think that's a great starting point for this work.

10:18:22 And at the same time I am going to try and spend a little time with some of the social issues, too, and a couple of things I've come up with that are very main focused for our conversation today.

10:18:36 Back in the day to do a genome sequence.

10:18:40 The first one costs around 3 billion dollars. it took a dozen years.

10:18:48 It's still it I think just recently they keep announcing there's sort of a joke in the genetics field where they keep saying, actually, we're done with the human genome sequence.

10:18:58 Now now we're really done. Oh, there was a part we didn't get to.

10:19:03 There has been another recent announcement that it is, in fact, fully complete.

10:19:07 The The machines themselves used to be these large, clunky, expensive

10:19:13 Now, you see, people are using genome sequencers the size of a USB. drive.

10:19:20 There have been applied in emergency room situations and it's it's essentially when I say genome sequence.

10:19:28 What I mean? is an analysis of your a's and t's and G's and c's the little chemical combinations called nucleotides, that make up our dna and this you know again this this this idea that

10:19:47 it's now quicker, faster. full like clinical grade. you know something that your doctor would be able to interpret and use is now going for a few $1,000.

10:20:00 The first one like I said. It costs so much money.

10:20:03 There was initial, you know there were days where it was a 1 million dollars half a 1 million

10:20:09 And now it's it's really quite inexpensive when in in the context of of medical care, like I said today, we're gonna do it Focus on medicine and help and a little bit about society I don't know how

10:20:25 much ancestry testing, and some of those questions are the work of this committee.

10:20:29 I, I get the sense that it might not be So this organization, Usp: 7 is a founded by a family here in Maine.

10:20:41 The big Ao. family in Falmouth they have a daughter Tess.

10:20:47 She's the in the second row third one over little cutie with the glasses smiling.

10:20:54 Their experience. is it's it's rare in some ways, and in in other ways.

10:21:01 You know, having a rare genetic disease, is quite common, and in their case test was, you know, they had a baby who had a mystery.

10:21:13 Symptoms, you know, developmental delays things that you know.

10:21:18 They were struggling to find answers, for they were eventually able to get her genome analyzed.

10:21:25 And one of the this is sort of this amazing story. where they got the variant, and no one.

10:21:31 It's incredibly rare. There was virtually nothing in the literature and the dad bow told a friend someone posted about it on Reddit.

10:21:40 The the message Board, and you know my daughter, Has anyone heard of this?

10:21:47 What is the life expectancy? You know they knew nothing right.

10:21:49 They knew nothing about how long like what her trajectory might be.

10:21:55 And with in a day they got a call from Baylor Research.

10:22:00 You know, University saying we there are others tests is not alone like there are other kids with this, and you know they have become activists and fundraisers.

10:22:10 In 2,022 I ran into. I ran into bow a few weeks ago at a lacrosse field.

10:22:16 There are now a 130 patients they found all over the world, and it has a name.

10:22:22 How fountain syndrome they're raising money funding research.

10:22:27 It's quite an amazing story. and many people who you know have rare genetic conditions become activists right?

10:22:36 They become their their own advocates and activists and it sounds like we'll hear about some of that later.

10:22:41 Today. movie star, Angelina Jolie announced that she carries a variant called brca, one which increases dramatically her risk of breast cancer. Right?

10:22:56 So she sort of went public, was saying i've used genetic information.

10:23:02 You know I got this information that i'm using to make pre like i'm using it as preventative medicine.

10:23:07 I guess, is sort of the best way to think about her story.

10:23:13 Many people are excited around genetic testing because of the potential for it to help people choose prescription medicines more safely and more economically.

10:23:28 I don't know if any of you have had this experience but many, many people who take prescription medicine an enormous percentage.

10:23:35 It doesn't work as intended the hope is once you get a picture of say, how do I metabolize this drug versus that drug?

10:23:46 You can prescribe more efficiently in this example.

10:23:52 We use. This is about coding metabolism.

10:23:56 We are organization focused on that because it's a commonly prescribed drug for teenagers and children.

10:24:04 Who are having like wisdom, teeth extracted also it's sometimes used recreationally, and so we wanted to sort of say, you know most people have a fine outcome, when they receive more.

10:24:15 Feeding, but some people don't some of you may be familiar with the drug warfarein, which used to be quite commonly prescribed. I don't know if it is anymore.

10:24:27 That's another drug where you can take a simple genetic test, and it can tell you something quite important about dosing. and, you know, reduce negative outcomes.

10:24:36 So like these are the things people are broadly excited about.

10:24:42 I did make a note on this slide. because you know, one of the things people think about, and and from a legislative standpoint is, you know, people want to know that their information is protected and private to that end in

10:24:57 2,008. There was a Federal law pass called the Genetic Information Nondiscrimination Act.

10:25:05 Advanced by our Us. Senator

10:25:15 Olympia. Snow

10:25:19 No, thank you. A little bit. snow in the Senate, my louis slaughter in the house signed by George Bush in 2,008.

10:25:28 It extends protections to people of their genetic information in 2 realms.

10:25:35 Health insurance, which means a health insurance, cannot take your genetic information and use it to raise premiums.

10:25:39 Lower premiums deny you insurance it's also protects people in the workplace.

10:25:45 Employers cannot ask you for genetic information, for hiring, firing, promotion.

10:25:51 Do you motion, etc. there have been a handful there haven't been many cases brought to sort of test like How?

10:26:01 Where does like, How strong is this law? But there have been cases.

10:26:06 There have been judgments in favor of people who said i've been, you know, when I when I shared I had

10:26:13 I was caring for someone with huntington's or I have got a the type of breast cancer that's a brca informed.

10:26:21 You know there were There have been lots of people who felt they were being discriminated against, and and were found that you know that they found that that there were judgments supporting that

10:26:34 One of the things i've been keeping my eye on is some States so Gina protects like I said, health insurance and employment.

10:26:43 But there's other types of insurance long-term care disability.

10:26:49 Gina, as we call it, does not cover those, but some State legislatures have enacted extended protections.

10:26:58 Florida being one that they have a this law that they have sort of extended this idea to long-term care, insurance, and disability insurance.

10:27:10 There's a lot of companies in and like from insurers about this.

10:27:18 Yes, So thank you for working with me when the bill that I introduced in order to plug this loophole in the State, because Florida is the first to enact this bill, and Maine would have been the second

10:27:34 but hopefully, the bill that would plug that whole and disallow insurance long term healthcare long term life insurance and and and other instruments of insurance hopefully that bill

10:27:54 will be reintroduced in a future and if anyone's interested, because i'm termed out in taking that on.

10:28:02 I will be happy to talk with you about reintroducing that for the 130 first.

10:28:07 But thank you for working with that. that was me okay great I didn't. I didn't even I I will admit I didn't

10:28:16 I don't think I knew about that so yeah so so you know there's like I said there's it took a long time to get Gina past.

10:28:24 There was, you know, many different interest groups have different views on these things.

10:28:30 But thank you for sharing that like I said I didn't know

10:28:36 And then you know the other area where people and where many, many people encounter genetic testing for the first time is in the course of pregnancy.

10:28:44 And genetic testing has advanced quite dramatically in this regard, that now there's a type of testing that's quite commonly offered to people called N.

10:28:56 Ipt non-invasive prenatal testing and involves analyzing a blood sample from a pregnant person to learn about the traits of the fetus.

10:29:04 This can be done at as early as 10 weeks. One of the things in our travels talking to people all over the country is, of course,

10:29:15 We know that the moral status of the embryo of fetuses, you know there's many many viewpoints from all walks of life that have a lot to say about this I've certainly run into folks

10:29:27 who have said I would never you know there's there's anyway?

10:29:31 What I I guess what i'm saying here is you know this sort of analysis.

10:29:38 We're not talking about changing dna we're looking at what you know, what the traits that exist in the fetus are.

10:29:44 In some cases also the embryo. there is a technology that at this point has been around for decades where you know, you can screen embryos as well at a sort of 3 to 5 day stage where you can

10:30:01 remove one of the cells and analyze it for certain genetic characteristics.

10:30:10 Many George Daly's piece referred to this technique, pre-implantation, genetic diagnosis, part of what his argument in that article was. that he was arguing that we may want to get You

10:30:25 know he was sort of arguing in favor of altering the Dna of an embryo to prevent you know, if there's a single gene often condition that they could repair it at this stage there's almost

10:30:45 nothing more controversial than this subject. in the field of genetics.

10:30:50 But part of what he was saying is, this technique is not always as successful as people hopes.

10:30:58 It would be where you analyze an embryo.

10:31:01 And then, from that analysis decide which ones to transfer to implant into the wool.

10:31:12 There's you know, he cited some statistics?

10:31:15 I thought to myself, what are the other statistics around?

10:31:16 How well, how successful is it, you know I mean there was I mean that's always the way with opinion.

10:31:23 Pieces. I always appreciate the different opinions and So So again, this, although this technique, like I said, this is been around for a long time like like 30 years lot of people haven't heard of it and again, I think when we think about

10:31:41 our work around genetic engineering and genetics in our lives There's so much work to be done in engaging people on.

10:31:50 You know where, where these sorts of things fit in their minds, in their lives.

10:31:57 In addition to the human piece I wanted to talk a little bit about you know we've you know, some of you may have seen some headlines around because we have amassed so much genetic information about ourselves through through companies like the ones

10:32:09 I mentioned which are, you know, you pay a few $100

10:32:16 There's a world of people who are so interested in this subject, and genetic genealogy that they have built like open source databases which then can be surveilled by law enforcement in an effort to try and

10:32:31 solve cold cases. So in this case you know, they someone had built this company called Jed Match Open Source.

10:32:47 It was used for people who interested in genealogy.

10:32:49 Why wanna find the roots of my family? An investigator in California?

10:32:55 Thought. Hmm! it's interesting and got some dna from this This you know, they there was Dna of a perpetrator left behind.

10:33:06 They were not finding anything in the Federal databases like codis that it's overseen by the FBI, and so they, they you know, made a fake profile uploaded it, and said do we are there, any

10:33:20 matches. and sure enough, you can hire a genetic genealogist. that is, somebody's job who can then build out a family tree and say, all right.

10:33:30 We think it's going to be a man of this age in this location.

10:33:35 You know you can find people. This happened in Maine last 2 years ago.

10:33:42 A cold case where he, you know he was working as a nurse, I believe, for many years.

10:33:50 And through this sort of familiar searching I believe he has been since convicted, and is is appealing the verdict

10:34:11 One of the things we've been talking about is the ethical question.

10:34:16 Certainly one of the big ethical questions is about cost and access.

10:34:19 This is simply not not, you know. Can I access genetic services?

10:34:25 Can I access genetic medicine? But can I get my basic health care? right?

10:34:30 So this is sort of an older slide. but you can see that many, many people are delaying here.

10:34:37 Certainly the pandemic has has likely accelerated a lot of this, so you can see there are many issues to to think about.

10:34:48 I don't know if any of you saw the news recently about

10:34:53 There's a new drug newish to treat sm a spinal muscular atrophy so Jen.

10:35:02 So little Jens ma it's the most it's It's 2 million dollars.

10:35:08 You need one dose it costs 2 million dollars right it's a Crispr informed approach whoops clips on a gene. it's gets a gene doing Its job. as it hadn't been doing so.

10:35:30 I think these cost questions are certainly like a major topic.

10:35:35 For us to talk about. The other thing I just I wanted to sort of quickly.

10:35:41 Mention is related to access right. There are certain groups of people, certain communities that have felt left out targeted both and one example is the Navajo nation.

10:35:58 Where there have been a number of sort of situations where it felt like their Dna was being like taken from them under false pretenses, or the work that they thought they were.

10:36:09 You know the research. They thought they were signing up for didn't exactly turn out to be, or what happened and there have been lots of new like models where sort of protected.

10:36:23 You know where groups that have been marginalized or targeted have have sort of work to develop new models of consent.

10:36:31 This will be, I think, something to really think about as we think about genetic engineering and genetic therapies.

10:36:39 Another example comes to mind is the the book the immortal life of Henrietta Lax.

10:36:44 The lax family you know there's still there's like many lax descendants.

10:36:49 Who have healthcare needs who have had their their genomes sort of publicly broadcast, and even after the book there continued to be researched on that, the family didn't necessarily agree to they have since broken an arrangement with the

10:37:07 nih to have sort of this layer of oversight, because it's their own family's dna in those hela cells which are used broadly across the entire world

10:37:24 So when am I getting to Crispr right? What are you talking about?

10:37:29 Christmas, so I I wanted to sort of go ahead when you talked about cost.

10:37:37 I I know there's another group another panel that's looking into the cost of medical drugs.

10:37:45 I I just vaguely in my mind, because the issue is, if main Care has a patient like an sma child born.

10:37:56 What will be What will we do and so I think there's another group, and I wondered if for the next time we could hear about that group.

10:38:06 What they're doing with their charges. just so we know that kind of Yeah, I I personally would love to know more about that, too.

10:38:16 Because I think right. this this idea of of access, right as as the Fda starts to approve some of these gene therapy approaches, you know. I mean, I think, for the sm a drug frizzle I gotta I have a

10:38:33 written down. I gotta figure out how to pronounce it.

10:38:36 Here Go, jansma to janza They there are some. there's almost like a one of the things I know is that there's a lottery for access to it which again you know so much of what we're

10:38:50 talking about today, and I should have said this earlier is deeply like personal and that we're talking about like you know, human suffering and families that have endured all sorts of difficulties and losses in many cases and so I just

10:39:06 want to say that when i'm like teaching and talking and if we go forward in some public consultation work, everything I talk about I work on the assumption that i'm talking to a room full of people that have been personally

10:39:20 impacted by it, which I think has has helped us create some.

10:39:28 Some good models around how to engage people is, you know, thinking about how how personal this is.

10:39:39 So whisper.

10:39:45 Clustered, regularly interspaced, short, palindromic repeats: Yeah, this is not helpful for most people, and in my personal view, I like, I think, what I was hoping to sort of we could sort of get to today.

10:40:07 Is like we can talk a little bit about the sort of detailed molecular business of this. Huh! Let's do it.

10:40:16 Maybe you can do it. but what what I wanted to sort of get at is like a real like sort of the lay person understanding, which is essentially everything.

10:40:28 I talked up before Was genetic analysis right? What are the genes I have?

10:40:32 What is my pattern of at T. Gcc. Cc: Right?

10:40:37 Crispr is this is this: basically it is a a model like a tool that allow scientists to make a specific targeted changes in your Dna.

10:40:55 So where you had an at Tg: Right you can. This This tool is like like a scalpel, like a molecular scalpel is one way to think of it.

10:41:08 Where you could say I wanna go out that exact at tg That's what I want.

10:41:15 I can like program my like heat seeking missile is how I think of sort of the cast 9 protein to go, and it chops, and it's incredibly specific and accurate.

10:41:32 It has worked in every organism that it has been tried in plant, animal bacteria virus.

10:41:40 You name it, and when you go after that particular site, that particular target, 2 things can happen.

10:41:53 You can chop out that section that you didn't want and because Dna is amazing.

10:41:59 It will. the ends like if you cut here, and you cut here, it can repair itself, or can reconnect, or I that at Tg.

10:42:15 I need it to be something else. I need it to be.

10:42:17 Gc. Aa. So you can send in like a like a replacement template, and it'll copy that new template in

10:42:31 Here's a picture

10:42:35 This to me is the simplest picture that can sort of give you the idea right?

10:42:40 So. So there's 2 things right there's our this is our double helix.

10:42:46 This is our Dna and Crispr is typically going after the point in the Dna. that code for proteins like the the sort of the active in a way that's one way.

10:43:03 To think of it. Someone else has a better metaphor.

10:43:05 Feel free to unmute yourself. And And so this those bright lights were the where you know the 2 dots.

10:43:12 Those are the cuts. enzymes. do the cutting so like high school biology, some of you are like, Please stop no

10:43:25 And essentially, what you can do is either chop out that pink piece, you see, and send in a new one.

10:43:32 So if you have a gene that is promoting the growth of cancer cells, you don't want that to do that anymore.

10:43:41 You can't. chop it out and put in what is sort of considered the the working like the the more preferable version of that sequence sometimes.

10:43:57 So you know you Also, if you just you don't need it to you, just want it going, and chop and orange piece one and orange piece 2 they were. They join up.

10:44:06 They repair themselves

10:44:16 I have a video. Where did I we'll get to The video? So remember, before I was talking about screening fetus or screening an embryo, What if what do we have here?

10:44:36 Right. There have now been advances in the field where we are altering. Dna using Crispr, or I mean Crispr, to be honest, has become kind of like a a shorthand.

10:44:53 There are other types of genetic engineering talons zinc finger like there's different words for it.

10:45:02 Crispr, I think, is probably the dominant technique in part because it's can be so specific.

10:45:10 It's worked very well. like for example. at the the Jackson lab has a summer program, you know, where high school and college students come every year.

10:45:23 They're all doing Crispr like this goes on in in high schools colleges.

10:45:31 Obviously the students at the jackson lab are quite extraordinary and it's not to say it's easy, but it's It's widespread and it works you know it works with undergraduates and high

10:45:44 school students who are looking to learn about, you know, using biotechnology.

10:45:51 Are there any questions before I keep going

10:45:59 Yeah, you can. You can buy kids and do it at home.

10:46:05 Like you can order one on your cell phone, have it Come to your house, and you can do.

10:46:12 Insert things into you that change, or into an organism that change things.

10:46:19 So this is available. it's here I think that's one thing to understand is it's upon us, and of course, for many, many people This is incredibly welcome news right when I was talking to my principal investigator you

10:46:38 know the one that has tingu who has the lab you know she's been around a while, and I was like, you know.

10:46:45 Help me understand how big of a deal this is. You know.

10:46:49 There was recombinant Dna in the Seventys.

10:46:50 There was the genome sequence. in the 2,000 and she was like There's this is the thing There's This is the biggest thing in my 40 year career.

10:47:02 Probably might be longer than that. i'm not sure so that's all to say.

10:47:08 You know this has been. People have been waiting right? Layer Richards is this little girl she was born with?

10:47:14 I have some notes here, because my brain gets a little fuzzy.

10:47:20 She was diagnosed with the newborn, with leukemia.

10:47:24 It affects blood and bone marrow. and so, by the time she was turning one, all the traditional treatments had failed right, and so

10:47:35 Her family decided to sort of go forward into this new frontier to have she had.

10:47:42 I have. I have no to write this down donated immune cells that were genetically engineered specifically for her body and her type of cancer.

10:47:50 They call it immunotherapy. So it was sort of engineered to both bite her cancer and not trigger or immune response. Right?

10:48:01 This has been one of the major challenges in gene therapy is the immune response that that causes the the drug or the intervention to not succeed.

10:48:13 These cells. they're called cart t cells and they were engineered to attack her team cells, and then they were altered to ensure her immune system wouldn't wouldn't perceive them as foreign and it worked you

10:48:29 know the last that they were in the paper. Her leukemia was was cured.

10:48:35 One thing to understand. there's another so we've already talked about analysis and analyzing Dna, and changing Dna.

10:48:44 There's another thing to really think hard about I think which is altering the Dna of a person who, like Leila like that alteration to her immune self.

10:48:59 We'll stay with her for her life but will not be passed on right.

10:49:06 So this question of changes to like your soatic cells is the word the cells in your body versus your germline reproductive cells.

10:49:18 So there's this that's another thing that Dr.

10:49:21 Daley was getting into right in his editorial was the consensus.

10:49:27 There's more consensus I would say around you know intervening on.

10:49:33 I have this condition. I am a you know I we're gonna change the cells in my body, and then that I don't pass that on

10:49:51 There's a question for you all imagine one of these genomics companies, right.

10:49:56 They're normally self for a few $1,000. It can tell you about diseases for which you have a risk of

10:50:04 You have a low risk. You have a high risk. you know a free, genome sequence.

10:50:09 This is a few $1,000 worth of value. Who would want it?

10:50:14 Do you have any takers

10:50:22 Right. And so this this is the teacher. part you?

10:50:32 Yes, I think I would be interested mostly on the ancestry part of it.

10:50:35 Just coming from a community, being a descendant of slaves, and not really knowing where my family comes from.

10:50:41 The rest of it. not really sure how I feel about that.

10:50:44 But the ancestry part is definitely of interest to me.

10:50:50 Yeah, I mean, I think thank you for sharing that I mean It's It's a for people that have are part of sort of diasporas like the enslavement of of you know millions like that the legacy is still very

10:51:02 much with us around what is what you've can find in your Dna people looking to rebuild and under understand connections that have been like violently broken.

10:51:14 There are So So so. yeah so that's a major reason outside of the medical piece that would drive people to maybe take take up this offer.

10:51:26 Any other, any other perspectives to share before I continue yes, oh, i'll speak for my sister.

10:51:34 She was adopted, and so she was interested in finding out her history.

10:51:39 So she did this. found out. She had 7 brothers and sisters off from the same mother all different fathers.

10:51:46 Yep: Yeah. and she has fetal alcohol syndrome right?

10:51:53 So people find things. so. some people go looking for information. Some people get information that they were not looking for.

10:52:02 That is a very common experience. and so okay so we've got some.

10:52:09 We've got some anyone else has something to share should I keep going

10:52:18 No, I don't you know my my grandmother died fairly young of cancer. and I'd like to know if I, if there's a genetic component to that that I may have inherited and if there's anything I

10:52:30 can do about it, and if not i'm gonna live life to the fullest, while I'm young you're younger.

10:52:36 Yeah, but I think it would be helpful plus i'm curious.

10:52:43 Sure, you know I think family secrets are certainly a theme.

10:52:49 And and one of the things you know, at least I have seen this many times over.

10:52:55 Right is certain types of cancers. Families are kept you know People don't want to talk about them right. their stigma, their shame to all sorts of things, and so you could not know someone who you grew up with that had this

10:53:09 particular can. you might not know So so thank you for sharing that if it got more specific and given the challenges of matching people to medicine, you might.

10:53:28 I could see an advantage to understanding the genomics around the administration of certain medicine to certain people. Yeah, if I was one of those people taking drugs, that was 50% likely to work 50% not likely to work, or cause

10:53:44 harm. i'd wanna have that testing before I started that drug, instead of flipping on coin requiring, genetic test before a medicine can be prescribed.

10:54:02 For this exact reason there's been some pushback on that, because in part with that that could mean, you know, is one thing that might happen is the blockbuster drug that everybody takes that might not be the case anymore.

10:54:21 Once you can prescribe it more narrowly based on a genetic But again, like I said, there's this.

10:54:32 This is like a vast last topic. The next question is, what if you are offered in addition to the sequence?

10:54:40 You know the analysis, the ability to make corrections.

10:54:49 Right. It's Free. Volunteers. it's essentially what that what I what this is codedly saying is it's approved by the Fda but there's no long term study, which of course is the precise situation many

10:55:05 people are in are diving into using these new technologies for themselves and their children.

10:55:13 I mean this idea of we're going to create your things is not quite it.

10:55:18 But obviously people who are going first in this age of genomic medicine are our pioneers.

10:55:25 Certainly so. Any takers well I I don't know if I would.

10:55:34 I think it depends on the situation. i'm I'm.

10:55:39 I I distinctly remember my very first year of medical school.

10:55:42 There was a before Crispr was developed. before we could really do any precise editing.

10:55:49 There was a family that came and We were just we were learning about Huntington's disease hunting since these. but those who don't know is a

10:55:57 It's an early onset dementia causing disease it's devastating It's also you only need one copy of it.

10:56:04 Unlike some conditions where you need to, to copies of it in order to develop the disease.

10:56:09 One copy means that the person develops this and it's and it always happens, In other words, this 100% penetrate.

10:56:15 So this family came to speak to our class in a lecture hall, knowing that it was just.

10:56:22 It was for the benefit, you know, for educational purposes, and shared their family history, and you know they shared their family tree, and who was affected and and they There were younger members of the family young young adults who

10:56:40 were had had chosen not to know their genetic sequence, because if they they knew it, they they would know that they were going to develop this.

10:56:50 If they you know, lived to a reasonable age and they didn't want to live life like that.

10:56:57 I wonder between your last question and this question now that there's something that can be done potentially. I don't and i'm not.

10:57:05 I don't think hunting pins is quite had like sickle cell. anemia has had tremendous already. 6 but I don't think hunting through this quite there yet. but I I feel like it's kind of waiting in the wings

10:57:16 and I wonder if if that would change their minds and and I know I've wrestled with that question.

10:57:24 I don't know whatever I would have answered then or or now, because it there, there's all sorts of ethical questions and questions about what does it mean to live life to fullest like Brooklyn neverado saying What does it mean

10:57:37 to what is living. These are really profound questions, and and difficult to put into the context.

10:57:44 But I think something has to do with there being a specific condition situation that there's nothing else that can be done for, and that it's likely to you know that there's it's not just generally finding out something like there are

10:58:04 so many brave people who are who are willing to go first.

10:58:12 Yes, yeah, as the the parent of a of a child with a very devastating disease that could be fixed by Crispr.

10:58:22 And i'm absolutely sure that at some point There will be I mean they're already in the pipeline.

10:58:27 They'll be they'll be a cure for this with the last question.

10:58:33 Had they tested me for this did I gave this to my son.

10:58:36 It's on the X chromosome and it undoubtedly came from me.

10:58:39 Had he been a girl, he'd have had 2 x chromosomes, and would have been a carrier, but wouldn't have had the full.

10:58:47 The Vol. Monty here so if I had been tested. I never would have known that I would be giving this disease to my son, because it's not in my body cells.

10:58:58 I've been tested and I don't i'm not a carrier.

10:59:01 So even it would have given me kind of maybe a fault sense of security that maybe I wasn't going to pass along anything devastating to my kids.

10:59:12 But then, on the flip side with this question, if somebody offered to correct the the disease, related Gene that I handed to him.

10:59:22 Well, yeah, I would. I would do that. I would I would sell everything I own and live in the cave for the rest of my life for that right?

10:59:30 That's how that works yeah thank you for that, and like bringing that like perspective around, like you know these like When I was saying about how some of these connections right?

10:59:43 These biological connections. We have you know, and and and it's. Obviously, you know you're part of a a community of parents that are, You Would you know, that struggle with these questions, and and I think more and more people as

10:59:58 genetic testing becomes more and more common you know there's Now we screen newborns for 40 or 50 conditions. right?

11:00:07 But there's research to do full genome sequences you know they're offering full genome sequences to newborns.

11:00:13 So you would carry that with you for your life, you know, that becomes part of your medical record.

11:00:20 These are still happening in research protocols you know and So there's a lot of questions ahead for for people that have that are that are younger.

11:00:31 I think, you know, being a neurologist, I diagnose and treat diseases that start and then carry through a person's lifetime.

11:00:44 And so i've had conversations like this with a great number of people, and I find that there are therapeutic nylists and therapeutic adventurers, and and some people will say I don't want to

11:00:55 have that unless this there are it's proven to me that that it's safe through my life time, and i'll just kind of keep watching this, and i'll you know give them an update when I see them year after

11:01:06 year, and there are other people who jump in and say, I want a better want to try to have a better lifetime.

11:01:11 Now this is my moment, I I wanna do something for other people who are come after me, and i'm all in, and so they'll agree to the research and and be all in and there.

11:01:25 You know, courageous, but I I understand both sides to it.

11:01:28 And so it really is a very individual risk analysis that people go through related to who they are.

11:01:36 Their risk tolerance, the disease trajectory itself what it is that it will compromise through their life.

11:01:43 Who is going to be compromised by it and So you know that's where counseling, and and just listening and finding out who the person is. is.

11:01:55 So on. Thank you for that, and I think you know one of the things I've again not an expert, but genetic counseling is a specialty that very much needed Maine has at when I first when I moved when I came here.

11:02:14 It. 14 or 15 years ago there were like 3 or 4 genetic counselors in the whole State.

11:02:21 Is that right? am I have I made that up no that's right so i'm going to assume there's more now.

11:02:27 But you know this is this is gonna be a big need, I think, in our like a workforce perspective.

11:02:34 And certainly there's also issues around how well genetic counseling is reimbursed by health insurance

11:02:51 I'm gonna come back. How am I doing on time my Okay, half an hour.

11:02:58 But you did

11:03:03 I'm gonna try not to take a whole hour i'm gonna come back to this video.

11:03:06 But I did actually let's let's see if I can show the video.

11:03:11 Now. so this is a very quick overview that talks about

11:03:16 We get to hear from Jennifer Dowdna, who is one of the pioneers in the field?

11:03:22 She wanna know when the Nobel prize a year or 2 ago for it.

11:03:28 Let's see see if this is embedded awesome yes, yes, alright. let's let's.

11:03:41 See here, let's see how I do with this I get this out of my way.

11:03:59 Because i've been looking at these home kits, and there's one called Crispr in a box starter.

11:04:07 Kit It costs 200 and something dollars, 169 is advertised as an educational tool.

11:04:18 $50 shipping. Sorry. Okay, So if you could show it from your screen, that would actually be helpful to me.

11:05:09 In 19,000. The sun's out

11:05:20 This is our continued search for the ever present technical problems lurking in the shadows

11:05:35 They're all

11:05:36 They're all university, because

11:05:58 I don't know if that's important

11:06:20 Why don't? Why, you solve this problem we take a 5 min.

11:06:24 Get up and move around break since you've been sitting for a bit, so we'll reconvene in 5 min

11:08:45 A science,

11:11:46 Yeah, she's

11:12:01 So we'll wait a second and see if you heard anything ,

11:13:33 Makes a probably for anyone on the So you want to turn your mic on it.

11:13:40 Want to turn your mic on for that. Alright, Anyone watching?

11:13:45 Thank you for your patience. we've been dealing with some technical issues, but we hope, cross your fingers that we've solved so we can resume

11:14:07 In 1987. A researcher discovered that some bacteria incorporate short pieces of viral Dna into their own.

11:14:16 The University of California is Jennifer. Douglas was one of the scientists who wondered why I love mysteries and I love puzzles.

11:14:23 I mean, I think that's why I became a scientist I love the idea that I spend my life trying to figure things out, And the mystery here was a lot of bacteria store pieces of viral dna their chromosome in their

11:14:40 own Dna. They grab these little bits of viral.

11:14:45 Dna, insert it into the genetic material of themselves, and then keep it for future use.

11:14:53 So why Downa and others determined that the bacteria are actually using the viral Dna Nice and future attacks.

11:15:03 The system is known by its acronym. prosper.

11:15:08 I kind of like in crispr's to biometric identification systems where you might store fingerprints or retinal scans as a way to be able to recognize specific individuals in the future bacteria are doing the same

11:15:23 thing, but they're storing little snippets. of Dna from viruses in their own genetic material.

11:15:30 But bacteria don't just identify their viral attackers by those snippets of Dna also devised a way of destroying them.

11:15:41 Conference in Puerto, Rico, Downa, and a French colleague, Emmanuel Chapantier, agreed to collaborate together.

11:15:49 They eventually figured out how bacteria do it.

11:15:54 Crispr can be regarded as programmable molecular scissors, so scissors that will be programmed to recognize a certain specific sequence on the genomes of the Atlantic cell and organism will be able to cut this

11:16:10 Dna at a very specific place. The Crispr systems and bacteria are really a seek and destroy kind of system.

11:16:21 They have molecules whose job is to detect that foreign Dna and recognize it as a virus.

11:16:28 And then, in the second step, Crispr molecules cut it up.

11:16:34 It was then that Downa and Charpentier made a conceptual leap that the history of science possible.

11:16:41 They want to extract Crispr from bacteria and take control of its guidance system.

11:16:49 In other words, could they re-engineer Crispr into a simple tool for editing any and any spot they chose?

11:16:59 After years of tinkering. they got their idea to work it's effectively, they realize this little molecular machine that can program it with Dna.

11:17:08 And basically that's why it's so amazing know a Dna sequence in an organism .

11:17:15 Can change that gene. Crispr is biology's super Swiss army knife.

11:17:20 It seems to be able to do just about everything, and can do it faster, cheaper, more accurately, more easily, than anything that came before it

11:17:39 So I like is a tool which theoretically could be further improved. So that Crispr itself determinology is really what we're using interchangeably with gene editing.

11:17:57 Yes, exactly that that and the other thing I I like about this video is there's a couple of metaphors.

11:18:07 Swiss army knife See can destroy, like whatever works for your brain.

11:18:11 You know, that gave you, I think, a couple of options to sort of start conceptualizing this.

11:18:16 There's lots of videos like this. one like you could watch 2 or 3.

11:18:22 Some are animated, some are for high school kids, some that that that documentary, the gene, is really quite a good resource.

11:18:34 I say that again with my disclosures We, my organization, worked with Pbs

11:18:40 To make their educational materials for their teachers. So I I maybe have a bias towards that documentary, but we found it very helpful.

11:18:54 It. It resonates with students. They do a lot of great work, sort of highlighting, like families and individuals who are sort of on the front lines of these issues.

11:19:03 So I wanted to go back to where we were. we were here, I think.

11:19:16 Okay. Good. So you know, I think i'm not gonna spend too much time on this, because I think we're gonna hear for some people later today.

11:19:28 But you know, cystic fibrosis is one of the conditions that there's some excitement around gene therapy.

11:19:36 Cystic fibrosis was one of the first sort of genes discovered, and it's like tied directly to a condition.

11:19:44 There was a lot of hope and excitement that discovering the gene would lead to treatments.

11:19:51 Unfortunately, that is not what happened. cystic fibrosis is characterized by like a like a stick mucus.

11:20:02 Build up in the lungs that makes breathing difficult as well as many other functions.

11:20:07 Sickle cell there's already. trials and and products underway in using Crispr to effectively cure single cell by there's different type.

11:20:25 There's 2 top main types of hemoglobin fetal hemoglobin adult hemoglobin fetal hemoglobin doesn't sickle it doesn't it

11:20:35 doesn't it doesn't get the see the picture the round ones right.

11:20:44 Those are considered a typical red blood cells, the long stretched out one.

11:20:49 Those are sickled. and essentially, when you have sickle cell anemia, those those they normally your red blood cells are moving smoothly and nicely, bumping up against each other, moving along nice and round the sickle shape They Get

11:21:05 caught. they put on each other. you know they don't move us freely.

11:21:11 So oxygen cannot be transported as well.

11:21:15 It leads to all sorts of issues around. You know what happens when your organs are not getting the right amount of eyes.

11:21:22 Oxygen regularly delivered to them single cell is is characterized by pain crises.

11:21:28 There's a lot of questions tens around because sickle cell is often seen in people of African Middle Eastern Southern Mediterranean descent.

11:21:45 Particularly though in America you see it most commonly in black populations.

11:21:52 There's a lot of criticism and debate around how research has been underfunded for sickle cell.

11:22:03 And so, without getting into the 2 to any details, I think we might have an expert coming on one of the meetings.

11:22:09 I thought that essentially there is a a crispr intervention that is basically looking to turn off the gene that is responsible for creating the self that sickle and turn back on the gene where the cells don't stickled If they

11:22:29 stay nice and round and i'm looking at the biology Professor in the room.

11:22:39 And that's again we could we can go back to this.

11:22:45 If there's some interest in like some real if we want to really get into sickle cell, and certainly there's lots of expertise, I can just make it clear that both cystic fibrosis and sickle cell disease are

11:22:57 devastating diseases. they devastate people's lives.

11:23:03 They shorten them, they make them uncomfortable day to day, and they're associated with crises that interfere with their function and the families function.

11:23:14 So I just wanted to amplify how you, how awful these 2 disorders especially are.

11:23:20 Yes, and and I know I have been sort of looking at some of like the young people in the sickle cell trials, who, you know, have suffered tremendously. right.

11:23:32 It's so painful many people with sickle cell are accused of faking their pain like being like drugs seekers, you know it's it's a real the the discrimination that many people who live with

11:23:47 sickle cell face. Is this like added like piece to an already like painful, difficult condition?

11:23:58 And so you know, you hear i've I watched an interview they 60 min profiled a young person who you know again a very brave to go first and saying, Well, i'm i'm happy to go first because this will maybe make it

11:24:11 easier for my younger brother, who also has sickle cell right like I'm.

11:24:16 I'll i'll Go first, i'll do it because I don't want you know my brother is much younger, and could, you know, suffer less?

11:24:25 Less years of this suffering and and the associated you know organ damage and other, you know, sort of negative impacts associated with with sickle cell

11:24:38 You mentioned that it's not it's not in the fatal cell. But it's more of the single cells more when they so can it be tested when they're in the fetus sickling yeah, because

11:24:52 there's a a trait That is inherited you know that there's the The trade is it may come from one parent parent, one parent, 2 sickle cell is a condition where, if you inherit one copy of the

11:25:08 sickling variant you're considered a carrier and you maybe will have you know you.

11:25:15 You don't have typically the sort of disease that we've just described.

11:25:18 But if you happen to inherit, you know, parent, one is a carrier, parent, 2 is a carrier, and you get the combination where you get 2 copies.

11:25:27 Then you have the disease. so it it can be tested.

11:25:33 You know in the course of a pregnancy you also screen newborns, you know, because sometimes you don't.

11:25:45 You don't know and actually this is a sidebar we now in America screen all athletes playing division, 1, 2, and 3 sports for sickle cell carrier, not the disease having one copy.

11:26:06 Cause there is. There had been a number of high profile deaths of admirals.

11:26:12 Where the parents in one case sued the school, and Ncaa saying, You know you.

11:26:21 He was exhibiting symptoms and that you didn't you know that they he wasn't treated properly.

11:26:29 And there's there was a lot of controversy and there still is about it, actually around.

11:26:35 Should we, you know, is screening all athletes getting the job done?

11:26:38 Or should we change how we do strength and conditioning, and to a day practices and a 105 degree heat?

11:26:46 So, anyway, but like if you any student playing any sport at the in Ncaa is screened for sickle sound right now to go sell treat I'm full of side bars i'm gonna keep going in this

11:27:02 case right? there was This is from a few years ago. but in a mouse model, which is, of course, where a lot of this work get started.

11:27:15 They were able to essentially cure a liver disease it's called type.

11:27:18 One tyrosimenia, and it's it affects one in a 100,000 people, and it's a single gene.

11:27:26 Mutation single gene change and essentially what they were able to do was use a Crispr system, you know, of of basically what they wanted to do was you wanted to sort of turn on a gene Essentially, the gene wasn't working they needed to replace it

11:27:46 with working copies, and they were able to successfully do that that once they injected the Crispr system into the liver, it was taken up by the liver cells, and eventually found in 33% of the cells in the liver which

11:27:59 was enough to restore function. I don't think this is in human trials.

11:28:09 Yet. but one of the things that has sort of quickly left into human into a human case.

11:28:18 R. Genome editing is also people are optimistic that you could potentially use it to end the global shortage of organs.

11:28:29 Pig organs are white, similar, some of them to human organs.

11:28:35 Scientists used a Crispr system to go after these particularly particular types of viruses that cause humans to reject transplanted organs from pigs and chop them all. out.

11:28:49 So this was one. Remember, we talked about Crispr. Sometimes you send in a replacement copy, and sometimes you chop it, and you get rid of it.

11:28:57 In this case it was getting rid of these particular types of viruses that trigger an immune response.

11:29:05 And just recently someone received a transplanted pig heart.

11:29:12 Part of the controversy about that My understanding was, It is.

11:29:18 It was not done in a clinical trial who clinical trials are.

11:29:23 Maybe that might be something our panel decides to take up around.

11:29:27 How widely available are they here and name? You know. How can we access them here?

11:29:34 Essentially, one of the things that happened that the part of the the bioethics folks will probably have more to say about this around.

11:29:42 What it means to to be part of to get that heart in a research protocol and outside of a research protocol.

11:29:50 You know what types of protections are afforded, what are the benefits of doing it one way over another?

11:29:58 I believe the person who receives the heart survive for a number of weeks or months, and then ultimately pass away.

11:30:09 Donor animal, and the gene editing done right, and one of the like.

11:30:24 The some of the companies that are like creating these pigs are actually like meet producers like the yeah, like the people who are like it's being like they're big I don't have the word i'm a

11:30:42 vegetarian, like whatever like industry, like large-scale farming of pigs.

11:30:52 The industries themselves are interested in and and funding and part of this research.

11:30:57 So when you talk about this to kids, they will say, could you still eat the bacon like from a genetically Mod.

11:31:06 If we, you know, genetically modify the animals to be good donors, can we still use the mean?

11:31:13 I don't know the answer but that's a question i've gotten from kids.

11:31:18 It's a good question actually it's a good it's a great question.

11:31:24 There's also. I sort of i'm gonna beer a little bit into some of the environmental questions 2, you know.

11:31:34 There's some discussion of using crispr to like alter insects that carry disease

11:31:50 I actually had an I had an interesting conversation with a friend from Angola when I was.

11:31:56 He mosquito landed on him he'd been just just newly here, and I said, Oh, you know the mosquitoes here are not carrying malaria, which is something that might be on your mind you

11:32:07 know but that they they won't give you that disease and he said, Oh, you think of malaria as a disease.

11:32:14 I was like I do. and he was like I don't you know, like it's like a these cultural questions around what we consider diseases like it's.

11:32:29 It was an interesting reminder, certainly and we'll talk a little bit more about some actually some like disability perspectives on genetic engineering, because there's a lot of activism in those inside of those circles.

11:32:42 But there's an idea that you could genetically modify mosquitoes to to no longer be able to carry malaria.

11:32:55 Well area again, is another massively devastating. I believe it is somewhere in the order of 400,000 people a year die from malaria around the world.

11:33:06 Still and this this caught my eye. there's a project in Massachusetts called Mice against ticks, and it's a like a community guided effort to prevent tick-borne disease by altering

11:33:23 mice and so I think we'll have plenty of time to get to this.

11:33:30 But I wanted to mention this in part, because this this and I should say, I know Jeanette Lynchoff and

11:33:39 I've known her for a long time she's a bioethicist.

11:33:44 But again, this work around can we use Crispr for sort of almost like what you would almost consider a public health intervention? right?

11:33:52 I think about lyme disease as a public health issue and there's the possibility of of immunizing the white footed mice which then are responsible for infecting tips.

11:34:08 This project, I believe, is is going on. on martha's vineyard, which has a massive lyme disease issue, and one of the models.

11:34:18 What? what grab my attention about this and Why, I brought it today?

11:34:22 Was It's a real like community driven process where one of the leaders of the organ of this sort of community work around, like, you know, Do we want to release genetically engineered critters here? on the island?

11:34:36 We all live on. is the high school biology teacher.

11:34:42 Who is, like, you know, a trusted resource in the community.

11:34:48 Where people, you know, have have you know and she's sort of proven to be an excellent leader for this for this work.

11:35:00 I want to go back to the humans just briefly to say one of the biggest stories that happened in Crispr was

11:35:10 When I was talking about before about there's nothing more controversial than changing the genetics of embryos right, or of like sperm and egg right things that are inherited.

11:35:26 And there is a right now, like in the United States, like no Federal money can be used to do that kind of research, but no Federal dollars go towards genetically altering vable embryos.

11:35:44 And and one of the things that happened at this summit was this scientist here announced.

11:35:50 In fact, he had sort of broken with these established norms, and, in fact used Crispr to edit

11:36:00 The genome of twin girls that were born in 2,018.

11:36:07 So you'll say the word claims here is doing some heavy lifting in this slide, because I don't know if, like the sequences of these children have been published, has it been this work. wasn't peer, reviewed when he

11:36:22 announced it. You know there was a lot of high controversy.

11:36:25 I believe What he claimed he did Was he altered a gene called C. C. R.

11:36:31 5 which is related to If you've got a particular version of it.

11:36:37 You are more resistant to Hiv infection than the average

11:36:45 This caused like an enormous It was an enormous incident in the field, because, like I said, this has always been sort of a red line altering embryos.

11:37:01 And there was a lot of you know there's again.

11:37:06 There's always sort of geography and race and politics that come into these kinds of stories, you know there was a lot of, I would say condemnation of you him personally.

11:37:16 He's imprisoned right now, I believe or at least was you know a lot of there was some backlash against China.

11:37:27 It's hard to you know it's it's a very difficult story to on on Tangle.

11:37:32 I would say. you know he trained in the Us. you know it is he.

11:37:41 The only is he the first are these like it's impossible to say really sort of where this stands right now.

11:37:48 But one of the things that you saw that sort of burst into the scene after these genetic modification, using Crispr of humans, right?

11:37:59 These girls are now 4 5, you know. Are they being studied?

11:38:07 Are they being Mod. Nobody that's not not knowable but one of the headlines you saw and you've seen now throughout, I think, is these questions of you know, is this the new eugenics right?

11:38:24 What's the difference between genetic engineering and eugenics?

11:38:29 Scientists confront the ghost of eugenics.

11:38:32 I wonder? Run a lot of time here. what? When we hear Eugenics?

11:38:41 What does that make us think of right cause this is we're getting into the real sticky part here?

11:38:44 Now. So i'm partially thinking, that that really was pulled in well with the conversations where everything about the bio ethics.

11:38:52 But if you could, if you could help make sure everybody on the panel under understands the term eugenics and a little bit of Maine history would be a big help, You're teeing me up nicely for my next

11:39:05 3 slides. so thank you for that. So you know I often will say the students. what are the fears?

11:39:12 Right when people ask questions like this, what is the thing people are worried about and students because they're like so they're the best, and they're so thoughtful we'll say things like i'm worried that I would be left

11:39:27 behind. i'm worried that I have a genetic difference that will be undervalued, that I would hate to think that it would be hurtful to me that my parents would have wanted to change me.

11:39:42 Others will say I have been suffering and I don't want this very I have, and I don't I I want the change, you know again.

11:39:53 This is this is like Why the conversation part is so important? Because the consensus part like it's not going to be a one.

11:40:00 Size fits all but part of you know the new eugenics right?

11:40:04 It's important to have a basic basic understanding of the old Eugenics.

11:40:09 So this was sort of a political and scientific movement in the United States that began in earnest in the early 19 Hundreds was you know, driven by a number of different things.

11:40:24 The industrial revolution, immigration, changing demographics in the Us.

11:40:31 The great migration of people leaving the Jim Crow South headed north for more opportunity, and you know less oppression.

11:40:40 And it sort of aligns also with some scientific leaps that were happening at the moment.

11:40:45 But the the idea is really was. It was a movement to improve society by encouraging some people to have children and discouraging other people to have children.

11:40:59 And you know it was you see here home for the feeble minded, filled with those whose parents I can't read that far.

11:41:11 My vision. Is not that good? I gotta read off my screen.

11:41:15 Home, for the feeble-minded is filled with those whose parents were not as carefully selected as dairy men breed Castle.

11:41:24 Okay? So This So this movement was like, why widespread in the in the Us.

11:41:35 And you can see here you know there were Arguments that certain people are desperate to become a burden, and you see, there's all these archival images collected at Cold Spring Harbor, which was sort of the

11:41:52 intellectual headquarters of the Eugenics movement is now a scientific research center.

11:42:00 So you know, you see, right like Some of these words more on, you know these are things that kids say to each other or people.

11:42:09 They were scientific categories. And And one of the the ways in which this movement unfolded was it eventually be?

11:42:23 You know that sterilization for sterilization was the tool and people who were targeted.

11:42:32 We're of all walks of life in particular if you were poor.

11:42:37 If you were had children, or we're having sex outside of marriage. gambling, drinking There was obviously a higher risk of of being accused of these things.

11:42:52 If you were black or brown. but not in not not always.

11:42:59 And this is the Supreme Court and 1920 can't read that far.

11:43:09 Oh, 27 you know this is the Supreme Court saying Society can prevent those who are manifestly unfit from continuing their kind.

11:43:19 3 generations of imbecils are enough. This basically gave federal power to sterilize people against their will.

11:43:27 So much like like there were eugenics boards the way there's like mosquito control boards in in places where there's a lot of mosquitoes you could be accused of being

11:43:39 of poor moral fiber of like I said. sex outside of marriage, feeble minded, was sort of a catch.

11:43:49 All phrase for sort of no conforming women.

11:43:54 And and you know you could be accused of whatever these crimes might have been, or or sort of deficiencies.

11:44:03 And the board, you know, would forcibly sterilize you.

11:44:08 This pedigree is from the state of maine and it's hard to see this far.

11:44:16 But you can see that the the categories, like what they sort of were arguing, were genetic traits running through families insane, more on feeble, minded alcoholic neurotic sex pervert.

11:44:31 We understand these are not actually genetic traits. Now,

11:44:40 Here in here in Maine, there's documented a few 100 for sterilizations, they largely happened at Pinland.

11:44:48 It's hard to say though you know so one of the things that sort of came characterized the eugenics movement is some of the record keeping, you know it's It's it's very difficult to to know there is

11:45:04 a professor. I looked him up. Professor Murphy at Usm.

11:45:10 Is considered an expert on pilot in particular, has written a book, might be a resource.

11:45:18 But I use this image from maine to sort of we didn't.

11:45:24 There were other States that had much bigger programs South Carolina, North Carolina.

11:45:29 Thousands of women they recently paid reparations to survivors I don't know.

11:45:36 I don't know what that movement if there's an a movement a foot here in Maine.

11:45:43 Don't know and these 4 sterilizations went on much later than you might think through the 60 seventies.

11:45:51 And in fact, there have been some cases. you know, in the 20, you know.

11:46:02 2017, 2014 where, you know, judges were offering reduce sentences If you agreed to be sterilized

11:46:13 I mentioned my sister, who has, you know, struggled with learning as learning disabilities, and my mother tell me that in the she was born in 1,956, that there were conversations about sterilization from my sister when

11:46:33 she came of age yeah, they didn't they didn't happen, but she told me that they were those conversations.

11:46:42 I think one of the things like, if we were to really sort of get into like a a broader discussion of this history.

11:46:50 Is a lot bubbles up for people. A lot of people recognize like events and their own family.

11:46:59 Maybe that they didn't quite understand but this has maybe helped them understand there's a documentary called Nomas Bay Base.

11:47:06 That sort of looks at the the sterilization movement particularly focusing on a group of women in Southern California who were Essentially, if you were poor and Latina, and in labor going into a hospital in southern California there was a

11:47:25 period of time where you would be denied care like active labor, no pain meds withheld until you agreed.

11:47:33 Unless you agreed to sign for your sterilization through the seventies.

11:47:42 So, and they brought a class action, lawsuit, and they did not win.

11:47:47 But there was all these changes that came of it including like this is why? like It's this class action suit like if you ever were to get a bilingual consent form or consent.

11:47:59 Forms now, and it's because of dolores magical in her and her Co.

11:48:04 Defendants in this case. So I wanted to bring this because again, right, what are we talking about here?

11:48:15 Right. it's good, I think, in my view to have sort of a handle on the old Eugenics.

11:48:19 So when we talk about these, when you hear these phrases, how how do they connect, and how do they not connect right like?

11:48:27 Sometimes they don't quite line up sometimes they do and sometimes they don't

11:48:35 The one thing I wanted to say this is a webinar.

11:48:39 I went looking for it, and we it's not online yet. But my colleagues and I we did a webinar called difference, not deficit reframing the conversation around genetics deafness and disability.

11:48:49 And I wanted to bring this today. We worked with a deaf bioethicist from call you debt as well as a geneticist from call you debt, and some teachers from Massachusetts, all of whom are

11:49:02 deaf sort of talking about you know. How do you?

11:49:08 How do you include the perspectives of people that Some people would consider disabled?

11:49:14 Some people would say, You know we could, could we? Should we?

11:49:17 Could we correct genetic deafness while there's a robust community of deaf people like capital D Def.

11:49:27 Who would say we don't know thank you you know we don't want these, you know this we are not in need of fixing

11:49:36 This is a massive oversimplification of the of the issues.

11:49:41 And there's a a I a massive amount of sort of scholarship around these these questions, you know, and I just.

11:49:50 I wanted to make sure that we we had. We could sort of hold this idea around the perspectives of people who you know around, you know, who would say like the nih is, you know, the the the part of the nih that studies deafness is

11:50:09 called communication disorders. Many people would say I don't have a decision, you know.

11:50:16 Deafness is a unique like there's a culture and language and tradition, and and it's not

11:50:23 It's not a problem it's a problem the world is not set up to necessarily communicate well with deaf people. but that's the world's problem.

11:50:32 And so I just wanted to sort of put this here, because I know we also are going to be talking about the urgency of families who are desperate for some of these interventions.

11:50:45 Would this be a good great booty? I think so.

11:50:49 We can put you as panelists for things agricultural Would this be a good breakpoint or lunch such that we could then put you on the invited panelist role in the second the second

11:51:15 session when we're, talking about the natural environment any questions before we before we finish, go ahead just I just have a really quick comment on eugenics.

11:51:28 It's part of my grad you Know to get my degree.

11:51:31 I had to take a class called history of genetics and we talked about Eugenics quite a bit, and it's been a long time.

11:51:36 But what's struck me was that those the eugenicists were highly respected, intelligent, kind of the elite members of society, and they sincerely thought they were doing the right thing no different than we sincerely think we're doing

11:51:53 the right thing with everything we do and so I think that's kind of humbling, and we need to keep that in mind as we proceed.

11:52:01 Yes, thank you for that. and I mean I think I think that's an excellent stopping point, right to sort of leave us to think about that, because it is right.

11:52:11 It was this came from the universities. This was at all the elite institutions, you know, when you took genetics you took Eugenics, but that was part of what was taught how it was taught.

11:52:24 So. so thank you for that point. I appreciate check our own hearts, and I assume all those Eugenics committees.

11:52:33 We're not diverse in our current understanding of the word they were all right.

11:52:39 Guys probably and some women there's a women as part of it i'm sorry to impute me, but no, there was. It was.

11:52:50 It was a relatively broad. It was a broader coalition than you might. that some of like the temporance movements, came.

11:52:59 There was some Eugenical thinking there. sort of.

11:53:04 But but yes, yeah, yeah, like I said I think there's lots of other.

11:53:09 There's probably some other people who have some expertise that I don't have that could enlighten us on some of them.

11:53:13 Yes, thank you, so well let's make sure there's no other questions of or very experience.

11:53:26 Capable teacher participants member thank you for that good Foundation.

11:53:34 That's been the intent of this morning was to get a good footing.

11:53:40 Oh, in a complex subject this afternoon we'll be hearing with from a number of people with different perspectives things to share.

11:53:50 Related to this topic, and get a little deeper into the science part.

11:53:56 So there not be any questions. I then turn my analysts and say, What am I leaving out other than remembering?

11:54:02 I use the mic before we turn for lunch.

11:54:09 Just to make sure you turn off your video will screen share of we're on, break slide for people to see who are watching.

11:54:18 But just make sure videos are off and your mics are all okay.

11:54:23 And so the lunch options are the cafeteria downstairs, which is a little skeleton, but it's locally packaged food.

11:54:32 It's not it's not produced in I was shipped here out of the spencer's with a full hour.

11:54:39 You'll probably get time to run down to the krishna brew up that way, or the what's the other one.

11:54:49 The other direction in Holloway there's a lot of choices around.

11:54:53 So everybody's got lunch on their own and we'll be back here to start again at one o'clock

13:19:25 Just to let folks know we're within a couple of minutes being able to get started after fixing some significant technical challenges will be a long shortly Dr. Shaw.

13:19:36 Your next up. Do you have a time by which you need to be done with us?

13:19:42 This morning was happening Senator i'm I'm working on that.

13:19:45 I will try to extend things outward as much as possible.

13:19:55 And what time in the ideal world were you planning to be done?

13:19:57 1 31 30. Okay, now, exiting the ideal world that's right.

13:20:05 But the ideal world is not the world we are always in.

13:20:07 So I I'm working with my team to make everything work. Okay, Well, thank you to you and your team.

13:20:16 I think we can go ahead and get stuff for him we ready to get going.

13:20:22 So we're reading this afternoon to Hear from some people with a particular expected perspectives on gene editing, and we'll start right off with Dr.

13:20:32 Nerves, Shaw and welcome him we've tried to set that up, so that there was 8 to 10 min of presentation and 10 min of time for questions and answers.

13:20:43 I understand that there may well be many more questions than are answerable in the 10 min, but that we need to collect those questions, so we can then prioritize them for subsequent sessions.

13:20:55 So, Dr. Shaw. welcome, and i'll turn things over to you very good.

13:21:01 Well, good afternoon, everyone. thank you very much. senator and members of the Advisory panel my name is Nura Shaw and I'm. the director of the Main Center for Disease Control and Prevention, and I am delighted to

13:21:11 join you for today's important discussion around the implications of genome editing technology for main right at the top genome editing comprises a group of technologies that give scientists the ability to change in organisms

13:21:27 Dna. These technologies allow genetic material to be added, removed, altered at particular locations.

13:21:36 In the genome there are several approaches, Jones editing that have been developed a well-known one. It goes by the acronym.

13:21:46 Crispr or Crispr cast 9. This is one that has generated excitement in the scientific community, because it is faster, cheaper, and more efficient than other methods.

13:21:55 For the purposes of my comments today. I will focus on Crispr, although I recognize that there are other methods out there, and will no doubt be newer methods in the future.

13:22:05 I'd like to focus my comments on the implications of genome editing technologies here in Maine along 3 axes.

13:22:14 The first are opportunities for research and collaboration. The second involve opportunities to improve the health of Maine's population writ large, and then the third are opportunities for a potential biotechnology sector.

13:22:29 Here. let's start with the first the opportunities to conduct research into these technologies.

13:22:36 Maine is home to some of the finest universities as well as world class research laboratories that infrastructure that already exists, comprising laboratory, space, scientific knowledge, and the ability to conduct cutting as cutting edge

13:22:53 research means that Maine has an opportunity to further research into genome at editing technologies.

13:23:01 Indeed, institutions in Maine are already engaged in research that can further out our understanding of these technologies.

13:23:09 Even though techniques and methods like Crispr, were discovered about 10 years ago.

13:23:17 In some ways we are still in the early stages of the potential for these technologies, and that means that Maine has an opportunity to position itself.

13:23:27 Now to be among research leaders. The second opportunity is the potential benefit for population health.

13:23:36 Here in Maine, genome editing technologies are of great interest in the prevention and treatment of human diseases.

13:23:44 The potential benefits for human genome and editing include faster and more accurate diagnoses, more targeted, precise and personalized treatments, and even the possibility of preventing certain disorders genome editing is being

13:24:03 explored in research and clinical trials for a wide variety of diseases, including single gene disorders like cystic fibrosis, hemophilia and sickle cell disease.

13:24:17 These same technologies also hold the promise for the treatment and prevention of other diseases like heart disease, mental illness, and infections like Hiv.

13:24:27 This technique could also improve treatment for a variety of cancers of particular importance to man.

13:24:35 Given our aging population one encouraging area is the use of genome editing technology for the treatment and prevention of devastating neurodegenerative conditions like Alzheimer's and parkinson's diseases

13:24:52 Now, though we should embrace these technologies we should do so with the recognition of the responsibility that such work entails as well as potential ethical concerns, it's important to note that most of the changes introduced with

13:25:10 genome editing are limited to somatic cells.

13:25:13 So manic cells are all the cells in the body other than egg and sperm.

13:25:19 Those 2 cells, egg and sperm are called germ line cells.

13:25:24 The changes that we've been discussing that you all have been discussing, and upon which i've been commenting our isolated only to certain tissues when they are done on the somatic cells of the body those changes

13:25:39 are not passed on from one generation to an X. However, changes that are made to genes in egg or sperm, so called germ line cells, or even to the genes of an embryo, could be passed on to future generations that

13:25:58 that type of genome editing on germ line cells, raises fashionable challenges based on these ethical concerns as well as safety concerns.

13:26:11 It is important to note that germ line and embryo genome editing are currently illegal in the United States, as well as in many other countries.

13:26:21 Another connection with any new technology is around health equity as such technology see even wider use across the United States and even within Maine we must keep equity.

13:26:35 Top of mind when we think about who can and should benefit from these technologies.

13:26:42 And finally, the third opportunity. The genome editing technologies present is the fostering of an even more vibrant biotechnology sector.

13:26:51 Here in Maine, and this dovetails with opportunity Number one that I noted around.

13:26:58 Research research is one part of the complex series of steps that are needed to turn technology into treatment.

13:27:08 Right now there is an opportunity for the biotechnology sector.

13:27:10 The player role in translating promising technologies into potential therapies through development, testing regulatory approval and rollout between our strong research infrastructure with world class institutions like Jackson Laboratory the University of

13:27:31 Maine, as well as a patient base that could uniquely benefit from these therapies.

13:27:37 Main, is well positioned to host advancements of this sort, particularly as it relates to the biotechnology sector.

13:27:46 In conclusion, among you are great minds that capture the promise that we have in our community.

13:27:54 Scientists, ethicists pass some quality language keeper, visionaries, the leaders of tomorrow.

13:28:01 People who navigate daily challenges in lives that could be affected and fundamentally changed via this technology.

13:28:10 Those elected who are with those who have been elected by the people of Maine to represent our interests, have an opportunity to do so.

13:28:18 I'd like to thank you each and every one of you for this important work that you're undertaking as well as your interest in the opportunities that could offer and improve public health if explored intentionally and ethically my

13:28:33 team, and I look forward to your report, and continuing our collaboration in partnership with you, Senator Quaxon, I will turn things over to you.

13:28:41 I have adjusted things on my end, and so, bye time is is more available, and I will.

13:28:48 I will leave it to you, sir. Well, thank you for that adjustment, doctor. Dr.

13:28:53 Shaw, and for highlighting the difference between germ line and somatic sales for those of us who are still learning our way in this arena.

13:29:01 Are there any questions about for sure at this point? hands up electronically, or wave hand in the in the room here, Representative Zacchar.

13:29:12 Thank you very much. I'll i'll on my appreciation to you, Dr. Schoff, for adjusting things.

13:29:19 Certainly, being here and always sharing your wisdom. My question was, you said that Maine has a patient base that you could uniquely benefit from Crispr or in Crispr type technologies.

13:29:33 Would you elaborate on that? Are you? Are you talking about things that where there was a founder effect, perhaps, or that could affect certain populations in Maine?

13:29:41 Can you please? elaborate? Sure. Thank you, Representative Zager.

13:29:45 You know I I had in mind there an expansion or another reference to our aging population.

13:29:52 A lot of the promise right now of crispr it's Some of the most ardent areas of research around it are around preventing or reversing the course of neurogenerative diseases.

13:30:04 Given our aging population the potential to have these technologies not just real and developed, but also deployed here in Maine.

13:30:13 I think it's something that is is really it is a potential like potentially quite exciting for us.

13:30:19 You. You will know far better than I the devastating impact that neurogenerative diseases have played particular on our elderly.

13:30:27 Given the nature of that population. We have an opportunity here to make sure that if these technologies are to be developed, Mayor is particularly older.

13:30:35 Manners can have the first stab at them. Thank you so much.

13:30:41 The other population jumps quickly to mine from the Lewis and Auburn area is the homozygous cardiac disease characteristics, and found in certain folks of Quebec Canadian

13:30:52 ancestry. Indeed, in the agreed, agreed Senator.

13:30:56 I mean there there are within any population main included there.

13:31:00 There will be pockets of those who could benefit first from proven safe ethical technologies.

13:31:09 The better we position ourselves. Now we have more opportunities to ensure that manners are the ones in line for those when they are developed and and found to be safe and approved.

13:31:18 Thank you other questions for Dr. Shaw. I don't see the Hope there go yes, or presume, Simon, hi thanks for being here.

13:31:28 Always about. I think it is really important to talk about the germ line.

13:31:35 That it's illegal to do federally to do germline research.

13:31:40 Could you expand on that. just a little bit more because I think that's really important, certainly.

13:31:44 So representative hymns, and I will. I will start by noting what I do not know, which is the the precise legislative history, and in the broader legal backdrop.

13:31:56 Around that I can. I can share where where, where things stand right now, and and my understanding of it, both in the Us.

13:32:03 As well as in some other select countries as I knowed and I think it's it's very important to reiterate.

13:32:09 There are 2 approaches, or there are 2 categories of cells in the body.

13:32:15 99.9, 9 9% of ourselves are what are known as somatic cells is our skin cells or liver cells.

13:32:21 Our heart cells the other. The very small percentage of other cells are so called germ line cells.

13:32:28 These are the cells that generate reproduction. Sperm and men in in males.

13:32:34 Eggs are over, and women almost all of the research.

13:32:39 In fact, so far as i'm aware all of the research that has been done to date, using genome editing technologies, whether it's Crispr or some of the predecessors all of that research has focused on approaches

13:32:54 to changing the Dna of these somatic cells. the 99 point, 9 9% of cells that is important, because, as I noted and i'll state it again, those cells, those alterations cannot be passed down in in subsequent

13:33:08 generations, however potential changes to germline cells, eggs, and sperm potentially could be.

13:33:17 Indeed, even in even in countries that have even thought about that idea of making alterations to Jeremine cells, penalties for those researchers have been quite severe.

13:33:27 So that is something that, as far as i'm aware around the globe is frowned upon, including in the United States.

13:33:34 The The precise legislative backdrop that brought those laws into place is an outgrowth of legislation that was started many, many years ago.

13:33:45 Many of us will recall the era of of cloning, and a famous sheep from Scotland, etc. that that era ushered in a series of laws at the Federal level as well as a series of

13:33:58 attendant regulations that prohibited that type of research.

13:34:02 What alone that type of conduct those laws have not been displaced.

13:34:05 And indeed, if anything, they've been replicated or rent in other countries around the globe, so put it differently.

13:34:11 I i'm not aware of any country's regulatory architecture according to the World Health Organization, that allows for the alterations to germ line cells, nor have I uncovered anything in my conversations or research that suggests

13:34:25 that there's any desire to change that and in in case It was evident, Representative Hamilton, i'll connect that 1 one step further.

13:34:36 This is a concern from an ethical perspective, no doubt.

13:34:38 And I think this is an area where the scientific, religious, ethical, and spiritual communities have taken a firm stand.

13:34:47 That that is a bridge too far, and and I think, to the extent there are ethical concerns that strong response by governments and regulatory agencies.

13:34:55 It it. It should give folks a little bit more comfort

13:35:13 Dr. Chester, Did you want to unmute yourself like I just unmuted myself, and join us?

13:35:19 Good question. So I don't know much about this but i'm wondering about the level of research on germ cells in heritable characteristics in non human animals, because I expect that in that realm.

13:35:37 There's been a good deal of research in fact maybe it's even the the norm to to work there.

13:35:45 So just kind of thinking out loud that the science is not really lagging behind, because black research they're the animal models right?

13:35:55 Really there's a a good deal known about Crispr and for germ cells.

13:36:02 Indeed. And, sir, I I can't I can't speak to the broader scientific background backdrop.

13:36:09 I I my comments could be You know my my comments are really focused on the human side.

13:36:13 But i'm certainly aware that there's a robust research going on on animals, and indeed a lot of plant research.

13:36:19 I don't know the extent to which that research is ongoing in the germline world.

13:36:23 I I don't want to misspeak on that so that we can add to the list of questions to think about being addressed when we get to the natural world discussions in our next session and we'll turn our staff loose

13:36:38 on doing some research on that in the interim, Dr. Chess on.

13:36:42 Maybe we can come a little bit better informed to the next meeting.

13:36:50 Are there other questions of folks either around the table or in the panel?

13:36:58 Well, I think. Dr. Shaw, that concludes for right. now. we reserve the right to invite you back again for sure we have questions, and hopefully at a time that's a little bit more predictable Oh, well, thank you all for the opportunity to

13:37:14 join you again. I I thank you for the rest of the undertaking and the work that you're doing. it's very exciting, and we we stand by to to continue the discussion.

13:37:23 So thank you very much. Everyone much appreciated your flexibility also.

13:37:27 All right, so I didn't really have it to take the time to set the format for the afternoon.

13:37:35 But we'll be hearing from a number of presenters this afternoon with their particular perspectives.

13:37:41 There's no schedule to break but we can certainly create one.

13:37:46 We're gonna hear from 6 or 7 people and I wanted to get to Dr.

13:37:49 Shaw as quickly as we could, because of his time constraints. But it's this point.

13:37:53 Not so any other questions we can proceed to hear from.

13:37:57 Jennifer Jul welcome thank you It's kind of humbling to go after Dr.

13:38:05 Shaw, but I was. I was glad to hear his comments, because I have some of the similar similar comments.

13:38:13 Thank you for hearing for me today, my name is Jennifer Jewel I'm representing the main chapter of the American Academy of Pediatrics, and I'm.

13:38:19 Also a hospital-based pediatrician, which means that I care for children who are admitted to the hospital.

13:38:25 You may think to yourself what a sad job seeing infants, toddlers, children, young adults, and adolescents at their most vulnerable, and caring for children while they suffer.

13:38:35 And die. I would like to remind you that modern medicine can alter the fate of many conditions that children and their families face, such as providing simple light therapy to prevent the devastating consequences of jaundice We can

13:38:47 even cure some conditions. like most pneumonia, with intravenous antibiotics and breathing machines overall the vast majority of children.

13:38:57 I I think I should put my video on i'm so sorry I forgot to do that.

13:39:08 Okay, Sorry. Okay, I apologize. overall. The vast majority of children are resilient.

13:39:15 And much of my job involves offering support and treatment while they heal themselves.

13:39:20 However, some genetic conditions lead to morebidity and often mortality.

13:39:25 Despite our best efforts, and our best science, many of the diseases that are amenable to gene editing are some of the cruelest.

13:39:33 I'm here to discuss 3 aspects of gene editing with you.

13:39:36 The first is the value of gene editing for patients like mine.

13:39:41 The second is safety, including possible side effects to patients undergoing gene therapy. And the third, and likely most important is ethical considerations of this new technology.

13:39:51 Here's my experience with 2 particular genetic conditions the first is sickle cell disease, which Dr.

13:39:56 Shaja just mentioned i've met many children with all of the complications of sickle cell disease, a condition in which a single gene defect that encodes a single amino acid causes red blood cells to

13:40:09 alter their shape, diminishing the ability to carry oxygen effectively, and clogging up tiny blood vessels.

13:40:17 These patients spend their lives modifying activities and taking medications to prevent the cells from sickling.

13:40:23 When this happens it results in painful crises, lung problems, difficulty, fighting, infections, and the dreaded complication of a stroke witnessing a toddler have the long-term outcome of a stroke is

13:40:36 particularly heart-wrenching. The second condition is final muscle, atrophy or sma a diagnosis that is typically made during infancy.

13:40:46 When gross motor milestones, such as holding the head steady or rolling over, are delayed. Within my first months As a position in Maine, I cared for a toddler with the most severe form of

13:40:56 Sma. I recall her face and her head of curly, sandy blonde hair, but it was her eyes that were most captivating because patients, with Sma lose their ability to move.

13:41:07 But they are cognitively intact. Wide eyes and their eyes have an indescribable brightness.

13:41:13 They use their eyes to communicate it's as if all of their life, and light and future reside in their eyes.

13:41:20 This child was admitted with pneumonia a common problem because of her inability to move her cough resulting in bacteria subtling in her lungs.

13:41:29 I was responsible for reviewing the options with the family, letting them know that we could likely treat this episode of pneumonia with antibiotics and an and an immediately inserted breeding tube that would

13:41:40 be connected to a machine for days or weeks, but that more frequent and severe episodes were certain to come.

13:41:47 The family decided to forgo the emergency interventions, opting for comfort, care, and their two-year-old daughter died in their arms. shortly after our conversation.

13:41:56 These 2 childhood illnesses are among many that are aable to gene editing because there are monogenic meaning.

13:42:04 They result from a single gene defect that scientists have identified.

13:42:08 Gene editing can be used to cure such conditions.

13:42:09 Saving patients and families from heartache, suffering, financial ruin, and guilt when diagnosed and treated early in life.

13:42:17 How far medicine has come that we can see a time of hope and joy and a future for these types of patients.

13:42:23 This is an awesome time to treat and cure disease, and we must admit that there are unknowns.

13:42:30 That is exactly why this panel is necessary. First, the panel may need to provide some oversight of safety concerns sure nearly every medical treatment has side effects.

13:42:43 Some side effects are easy to recognize and treat, others may present much later.

13:42:46 For example, a known side effect of some gene editing therapy is an overwhelming reaction to the treatment and patients.

13:42:52 It looks like a severe allergic reaction. A long-term side effect may be associated with yet additionally unknown functions of a gene that has been edited, weighing the risks of such known and potential side effects

13:43:06 based on their severity and frequency may become part of this panel's work.

13:43:10 The second reason this advisory panel is critical are the ethical conundrums associated with gene editing.

13:43:16 Many folks immediately consider the way that individuals and society may alter genes for enhancements or for engineering or manufacturing humans with particularly desirable physical and cognitive traits which would decrease the diversity

13:43:29 that we consider necessary for the health of future generations. I think about other dilemmas, too, like the experiments and animals, such as pigs that are being genetically altered, to grow organs that may someday be suitable for human

13:43:42 transplantation or the social justice implications for families that may not be able to access such treatments due to lack of money or knowledge or understanding, and the cost of the healthcare system.

13:43:54 I ponder the ways that researchers and scientists may inadvertently target minorities, populations on which to conduct their early gene, editing studies or neglect diseases that affect primarily minoritized populations

13:44:08 altogether. I commend Representative Zager for his foresight, and appreciate the amazing potential that gene editing carries.

13:44:16 However, with such enormous power comes the enormous responsibility by proposing and establishing this panel.

13:44:24 Representative. Zager demonstrates professional humility, the appreciation that some decisions, even medical ones, risks too much if left to proceed unchecked without oversight. thoughtful debate, diverse opinion, or full understanding These are the

13:44:39 concepts in the short term and long term that need to be reviewed by the panel, so that gene editing is accomplished with the best interest of manners as a population.

13:44:51 In addition to our individual citizens, I implore the panel to be confident, excited, and optimistic in the role that gene editing plays to cure childhood illness. to ask difficult questions so that gene editing is done

13:45:02 in the safest, most ethical, and most comprehensive way.

13:45:05 For all manners today, and future main generations. thank you for your attention, and thank you for your conversation to this important topic, or all these people wouldn't be here we did here I think so did you mention that 2 million dollars for

13:45:24 the therapy for that's the one That's the drug that we talked about earlier. old sold Jen Joel's gold agenda which does cost 2 million dollars must be given before the

13:45:39 child turns 2 but is my understanding i'll defer to your expertise, if and if you've seen this drug, I would love to hear about it.

13:45:48 That it? It is quite effective. Yeah, Thanks for the question.

13:45:54 I have I know of patients who have received it it's usually headed by our pediatric neurologist.

13:46:01 I don't know Dr. hammond if you've had any experience with it.

13:46:05 As an adult neurologist. it people are very optimistic about it.

13:46:10 And i'm glad that that was one of the the ones that you mentioned it is.

13:46:16 It is very costly, and I think, as part of this discussion as a society.

13:46:19 When we talk about gene editing we need to think about those those costs, and well, I don't think it's necessarily appropriate to put a cost on the life of a patient.

13:46:29 But but there is a whole system that that we operate in as providers as well as patients, that we need to be thinking about.

13:46:38 So we did talk about that some this morning. We also talked about sickle cell, disease and the study study or studies that are ongoing right now for potential cure.

13:46:53 Yeah, there. Yeah, Thanks for that question, too. There are there have been studies with with gene editing, and I think one of the important things to think about when we're just like talking about which conditions may be amenable to this one

13:47:05 of the questions should in my mind should be what are other options here?

13:47:12 Do we have anything else that would be possible and the answer for sickle cell is, there are a variety of different therapies being used.

13:47:25 They're all a lot of them are very expensive one people have talked about bone marrow transplant for for those patients as well.

13:47:30 I would say an equally expensive potential, equally expensive propaganda.

13:47:38 So. so I think I think the part of the point is, how can we do this?

13:47:42 In a way that is is more cost effective. And does that mean for pharmaceutical companies, or anyone doing this?

13:47:53 To to figure out how to how to make the cost less.

13:47:57 Will that, like other medications, Will the cost come down as time goes on?

13:48:01 But yeah, sickle cell is is one of the other. that diseases that have potential.

13:48:07 And there have there has been at least one person that I I not me personally, but I've heard about that has been cared from single cell disease.

13:48:15 Astounding. other questions in the room here, or from the panel.

13:48:22 Yes, Representative Zager. Thank you So much technical for coming, sharing your your experience in your heart.

13:48:31 What in your experience, what guidance can you suggest for distinguishing between enhancements and pathology or disease? That's a great question.

13:48:47 I think about that a lot I think in part an enhancement is something that's not would be something that's not pathologic.

13:48:58 That's not causing a medical problem, I would consider that an enhancement.

13:49:03 Some people would disagree with that but That's my opinion, so I don't think sorry Dr.

13:49:09 Claxton, but male pattern baldness is is a medical thing.

13:49:15 To be sorry to to be treated I I should have included you, too.

13:49:19 But but I would argue that that's a type of in hand, like we just know each other a little bit better so.

13:49:25 But I would, I would say that's a type of enhancement.

13:49:26 Some people may disagree with that. something that is truly medical requiring medical intervention.

13:49:33 I would consider that not an enhancement it does get very murky, though, when you think about things like, think about the deaf community and deafness, some some people consider that a medical condition a lot of a lot of people

13:49:50 don't they consider that part of normal that that's just part of the difference that that's not that's not a something to that's not not the same It's not a problem.

13:50:05 Yeah, it's nothing to fix you could talk about height too.

13:50:07 That Yeahand i'm gonna argue despite like you and I could be on that on that And i'm gonna argue despite like. So we have us having to ask people to help us in the grocery store to get stuff off the top

13:50:19 shelf that that would be an enhancement for most people.

13:50:22 Not maybe not all, but but just wanting to be a couple inches taller.

13:50:26 I'm not sure that that that I would concern about that enhancement.

13:50:30 Yes, Hi Nice to see you. yeah. there was a pediatric neurologist at the pediatrics meeting.

13:50:42 Yes, who I spoke with, and I think he would be a great addition to us.

13:50:45 Hearing based on his. You know his experience using a treating sma and

13:50:53 He. He is the neurologist who does a lot of the Sma treatments, and he also another reason that he would be very helpful.

13:50:59 Here is that he also treats a lot of patients. with discern, muscular dystrophy, and I know you're going to talk to a parent of of a Oh, okay, so it's it's

13:51:10 do you want? Am I supposed to say his name or just pass it on, or whatever. But But if you could pass it on i'm sure and I am he actually knows that I was talking, here and he would be very interested in

13:51:22 participating as well. that's great yeah I spoke with him too, at the meeting, and he seemed like he would like to do that.

13:51:27 He's wonderful

13:51:38 You know, the idea being, once you get this medication that you are now saving the person's lifetime expenses going forward of having the disease nursing home ptoot you know equipment everything would add

13:51:55 up to the drug company say much more than the price of the drug and so that's their you know that's the same. that's what they say.

13:52:04 And so the payment would be like a mortgage that you would pay it off over time.

13:52:08 I'm not sure. Yeah, I I don't know I wanna get into, you know.

13:52:13 Wrestling that out. but I will speak to the fact that this having these diseases can be very expensive.

13:52:19 So you talked about some of the obvious ones. The other ones are things like cost of being in the hospital.

13:52:24 Frequently cost of miss work for the family then there's the cost of home care, because a lot of these patients make qualify for like whether they can get it or not.

13:52:37 But they may qualify for around the clock care at home, and all of those are are definitely costs that could easily add up to more than 2 million dollars to treat.

13:52:48 Sm: Oh, sickle cell is probably the best.

13:52:52 A better example of that, because that's that would benefit over a long lifespan. The the the average age of somebody with sickle cell at least when I was back in training and i'm sure it's slightly better now

13:53:03 is around 50, 50 to 60 in in in our country, where we have for good medical care.

13:53:12 So you can imagine in other places it might not be that.

13:53:17 So when you start thinking about life, expectancy, you really have to be clear about what you're talking about.

13:53:23 So I'm talking specifically in in the developed world yeah so short.

13:53:28 So while it's shortened and there's all the costs associated with that it there there's morbidity associated with it.

13:53:35 In addition to the mortality and I think that's what the other thing that we have to to think about, not just like years of life.

13:53:44 But life life in those years, too. yeah I don't think of adding like adding years is having any value by itself, unless their quality nothing.

13:53:57 No question. you're you're and you have a academic appointment as well.

13:54:01 Any suggestions as we think about attracting retaining supportings.

13:54:07 Current future jet and feature generations of healthcare practitioners in Maine.

13:54:13 Any Any thoughts I realize this is not you, you know.

13:54:16 It may may be a surprise question, but we're trying to be as broad and as forward thinking as we can any thoughts regarding training or education.

13:54:26 For you know regarding gene editing yeah that's a little bit outside of the scope of what I expected.

13:54:34 But I will i'm happy to comment on if you'd like me to.

13:54:38 I I think I think that there is a need and I do think it's hard to attract in a small state people to who have that this very specialized area, having these discussions and putting this sort of at the forefront with

13:54:57 this panel will do nothing but help attract people here and potentially, and I I know there's like expanded training opportunities in Maine Now for neurology. It just happened in the last couple of years.

13:55:13 That we There's no training. in genetics for example. you could see a time where this sort of gene editing technology could advance things like that.

13:55:24 There's no reason to think that main couldn't be a leader in in some of this technology, and attract and attract more providers as well as other important pieces.

13:55:36 To this biotech companies, etc. Dr. Chessa, did you have a question?

13:55:45 Yes, I do, and let me lower my hand here before I forget

13:55:49 I mean it's it's maybe not gonna be the best form question, but it's good to see Dr.

13:55:54 Joel. my my my question is really about equity cause we're we're talking a lot about the expense of these these particular types of interventions and I I sort of want to place that in the broader context

13:56:08 of access to medical care generally, and you know this is gonna call on your sort of I.

13:56:16 You're probably your perception in your practice. But do you see an equity between public and private insurance in terms of access to see equity between folks who live kind of lower on this

13:56:37 socioeconomic. so scale versus higher on the socioeconomic scale. So sort of what i'm asking is, is how equitable is access to medical care kind of in general.

13:56:47 For our pediatric population. and and and how do we sort of think about as we're talking about these very expensive cutting edge technologies?

13:56:59 How do we also recognize, maybe, that even the more mundane medical care needs to be funded to to help these kids?

13:57:09 Yeah, I think. we live in a state, that is I think it's fair to say the the legislature recognizes the importance of care for for children, and that that we are fortunate that most children are covered

13:57:28 with with insurance. there's more to access of care than just having insurance.

13:57:33 However. you it's it's more than the card it is things like transportation. it's things like, Do you have a doctor who will take I think mostly we're talking here about main care and and and

13:57:49 so I feel like for primary care it's it's it's pretty good to at least in the Portland area to get in the door after what happens there.

13:58:02 It's a little harder to say and that's because there's all kinds of other things I I mentioned transportation and security. But there's also language barriers. there's all these other things and that's Why, I brought

13:58:13 it up, Frank. So thanks for thanks for recognizing it.

13:58:17 That a lot of these concepts you that you all learned about this warning are pretty complicated and explaining that to a patient and a family who's thinking it in your office about how am I gonna get home or I can't like I don't

13:58:31 even understand what the doctor saying, cause I don't speak this language things like that.

13:58:37 I think there there are large equity concerns and Then If you try if you're talking specifically about the genetic treatments.

13:58:46 They're very expensive, and so I I I would actually say, for main care to support, like all of that would be very, very, very difficult.

13:58:57 And so there, I agree. there has to be a thought about equity with this.

13:59:01 And and how we distribute this this technology? Yes, representative rata.

13:59:12 Thank you. I just have a I just want some clarification.

13:59:18 So when you're talking about genome editing technology, so to me, that means the entire genome.

13:59:26 But what you were talking about was more directed towards somatic cells, such as bone there on, and which which is legal.

13:59:34 And genome editing technology. My understanding is illegal and I just wanna make sure that we're the nomenclature is correct.

13:59:44 You you want me So so you bring up a great point, and some people, when they talk about a genome, they are talking about it. the germline cells, which is what you're saying, some people when they talk, and I actually like

14:00:01 in full disclosure. I thought the same thing When I was asked to do this I was like, I better load this up, because those are 2 different things.

14:00:09 So so. and and most people when they talk about genomic editing, they're talking about the both things somatic cells and and germline cells.

14:00:19 So the things that I was talking about, that i'm concerned about like the enhancements or manufacturing people with like an aptitude towards science, or something.

14:00:31 Though those would be more more germline mutations, and those are the things that I think have lots of ethical implications like like Dr.

14:00:38 Shaw said as well, so but you're but these these things that like circle cell disease, I was talking about sma

14:00:46 Those things can be done in somatic cells at at the time of diagnosis.

14:00:51 Starting around the time of diagnosis in in a baby that's already been born in this case. Right?

14:00:56 And thank you, thank you for that clarification and also with regard to cost.

14:01:00 Let's remember the computing power in my cell phone Let me think of how many millions of dollars that would have cost .

14:01:08 You know less than a generation ago. 1020 years ago and Now you get it for 100 bucks, and it would have taken up this whole room.

14:01:15 Yeah, So i'm i'm optimistic with regard to the cost.

14:01:17 I am, too. Thank you. That was one of the charges was to be Optimistic Riley, Representative Riley.

14:01:32 Thank you very much. I appreciate your presentation.

14:01:37 I just wanted to comment that when you talk about main care versus private insurance and access, and some of those barriers that you brought up that are really hard to quantify are spot on you know I've seen that I've ever

14:01:50 since my son was diagnosed 20 years ago. I have worked with other families.

14:01:57 In 2,003. We had an email list. Serve with about 300 families from around the world.

14:02:02 That were all do shen families that were exchanging information.

14:02:06 And then social media happened in the list. Serve went away. but those are the kinds of issues that are staggering for families.

14:02:14 In, and it can be things that are very, very difficult to quantify.

14:02:19 Every year or so my son has to be reevaluated.

14:02:24 For how many hours of care he can have, and that is always covered by main care.

14:02:28 There is no private insurance that will cover that. So, even though we had private insurance, he always had to have main care in order to get that care, so that I can go to work back when I was able to go to work and I had

14:02:41 a contact with another family. here in maine who's son was about the same age, and very much on the same level of disability.

14:02:49 It's a progressive disease keeps getting worse and So they were, but they were at about the same point needed help with the same things, and my son was eligible for about 30 h of care a week, and they got 10 and that's not

14:03:03 uncommon it's all over the map and it's very hard to quantify It's just there's no you know.

14:03:08 So. So when we're struggling, with those things I just want to make sure that that's out there front and center, that there is no baseline.

14:03:15 There is. No, this is how you do it it's Everybody's kind of throwing darts at the board.

14:03:19 I'm sorry that that's been your experience but i've heard it from same same from many people.

14:03:25 So thanks for being brave enough to, and courageous enough to to own it and talk about it.

14:03:31 Well, we've been fortunate so I feel like passing that good fortune along is kind of my my mandate, and that speaks to a bit to the issue of advocacy, and the decision making about all these

14:03:43 things. If you have 300 people worldwide advocating for something and 3,000 living in Maine advocating for something else, you're not gonna get the same amount of coverage support dollars that you that you might otherwise

14:04:02 any other questions or comments for Dr. Jul. Thank you.

14:04:10 So as an adult neurologist, I see the kids grown up, and so, and what they've been through, and then what follows them into adulthood?

14:04:21 And it's it's extraordinary what they have to go through i'm thinking of like tuberculosis man with tuberculosis, who has always lived with at least 2 caregivers because

14:04:33 he has violent tendencies and that's his been his life, and you know it has to be the same. he has to eat breakfast at 8 o'clock in the morning.

14:04:44 Otherwise he gets violent, you know so everything has to be very similar, and that goes into the group.

14:04:52 Homes, too, where people are don't have the ability to live in a single house.

14:04:55 But instead they're in a group home, and the challenges that happen in the group home with violence that gets pushed off to the emergency room.

14:05:05 So you know these are disorders that that take tremendous that follow people through a lifetime, and are just tremendous.

14:05:16 You know I think. What if this had been turned off when this person was, you know, a year old, you know, and didn't wasn't this other person?

14:05:26 What What would he be like as a person who's Jane had been turned off, and it allowed him to be a person without without this?

14:05:35 So it. This gives extraordinary opportunity. to people Yeah, that's a great point. and I also think about I mentioned in my comments about guilt. and I think about how the parents feel about that as soon as they understand that

14:05:51 This is some of these conditions. may have been transmitted through through them. And so you're just you're just pointing out the practicality of how difficult it is and to think about a family who there's all of those like

14:06:04 cascading events and the practicality, and then, like I did.

14:06:12 This to my child is is you know and we can say as as doctors that you didn't do anything, but I think it's really hard for some parents, and it it just compounded for for families as well so I I didn't mean to talk

14:06:26 so much. Thank you so much. We meant to ask as many questions as we could.

14:06:33 So thank you for being here, Dr. Jewel. we were scheduled to hear from Dr.

14:06:39 Zuckerman. but he's not able to join us today, so heavy, Hunter.

14:06:45 Well, if you would like to unmute yourself, and join us we'd be grateful.

14:06:50 Yeah. Hi: Thank you. I am actually a patient of Dr. Zuckerman.

14:06:58 I have a single gene disorder, so I live with cystic fibrosis.

14:07:02 It's otherwise known as cf it's a progressive genetic disease that affects the loans, pancreas, and other organs.

14:07:11 Cf. is caused by a change or a mutation i'm Sorry, excuse me, and a gene called cftr.

14:07:20 This genetic mutation makes it so that chloride can't correctly move to the cell.

14:07:24 This causes thick and sticky mucus to Sorry he looks like it.

14:07:34 So this thick and sticking you get primarily resides in the lungs and other organs.

14:07:40 This can then clog the airways and trap bacteria.

14:07:43 This leads to chronic and persistent infections information, and can often lead to respiratory failure within the pancreas and the gastrointestinal system.

14:07:54 The buildup of this new case causes the body to have difficulty absorbing and digesting food, which causes a lack of key nutrients for cf patients.

14:08:03 This often leads to malnutrition, and poor Grove, Cf.

14:08:07 Can also cause a multitude of other health issues, such as diabetes and liver disease.

14:08:14 Cf. has often had a very poor life expectancy throughout the years.

14:08:20 When I was born in 1998, it was suggested that I may not survive past high school.

14:08:24 Starting from birth, I have suffered from persistent lung infections and other various complications.

14:08:29 I would typically need to be in the hospital 2 to 4 times a year to treat these infections often 2 to 3 weeks each time.

14:08:37 I have had a more aggressive case of Cf.

14:08:41 At a young age I needed to have a feeding tube due to complications with digesting food.

14:08:47 Cf Patients really need to maintain a healthy weight as it helps their body fight off these infections.

14:08:53 I've also had many procedures to place. ivs so that my body could receive frequent antibiotics once again to help treat these infections in Middle School.

14:09:05 I also was diagnosed with Cfr. D. which is cystic fibrosis related diabetes, adding the management of another condition alongside Cf.

14:09:15 Has not been an easy task to navigate. Cf.

14:09:19 Has extremely demanding health Regiment, frequently leading to missing school, which in turn meant missing out on important milestones and experiences to manage my help daily.

14:09:30 My treatment consisted of many medications, upwards of 20 pills per day.

14:09:36 In addition to that, I also endured many hours of airway, clearance therapies and nebulaize medications.

14:09:43 This was to help clear my airways, clear my lungs with the ultimate goal of making breathing an easier task.

14:09:51 Regularly. These treatments needed to be done 2 times a day, and if I were sick we needed to be doubled. As you can imagine, managing my Cf.

14:10:02 Care has always felt like a full time job treating my Cf.

14:10:06 Has always been time intensive, and I speak for many cf patients.

14:10:09 When I say that many sacrifices must be made to try to stay as healthy as possible.

14:10:15 I was born with Cf. so i've never experienced life without it.

14:10:20 Experience. This, as a child, was a very difficult thing for me to navigate.

14:10:24 I often would have to explain to peers and friends what Cf.

14:10:28 Is Why, I always had a cough and why I wasn't normally at school.

14:10:32 One of the problems with Cf is that it can present as an invisible disease meeting those who have it don't typically look sick as one would think.

14:10:42 So these social social situations became normal interactions for me.

14:10:47 I had to grasp your own childhood at the time that I had to deal with where it's reflective of a normal experience to put this into perspective.

14:10:57 Now, i've had 23 years of lived experience with a single gene disorder, and everything that comes along with that gene.

14:11:07 Editing technology truly has the potential to revolution icf and how it's treated and managed.

14:11:12 I'd even go as far as to say that it's a potential pathway to eliminating the disease in the future.

14:11:17 Chris. we're technology as we've talked a lot about today has actually allowed for some researchers to correct meations that cause Cf and cultured human stem cells.

14:11:29 They use a technique called prime editing to replicate the full B piece of Dna and then replace that with a healthy piece as a Cf. patient.

14:11:38 I have followed the science and the different developments for years and it's truly exciting to think about all that could be done, not just with Cf.

14:11:46 But many other conditions as we've discussed a lot today. Speaking based on my own experiences, I believe that the pathway forward should consist of some sort of government system in regards to genome editing in the state of maine I

14:12:02 think a system like this should be a priority within the next 5 years, so that further research and development can be conducted in an ethically responsible way.

14:12:13 Do you know, i'm editing shows true promise for preventing or eliminating many diseases and conditions.

14:12:18 However, it's important that you're the pursuit of this that we are using the science responsibly in fair and ethical manners in the United States laws and funding policies at both the State and Federal level will govern human

14:12:31 genome, editing at all stages, from laboratory research all the way to clinical application.

14:12:40 These systems systems will be used to manage uses of human genome.

14:12:44 Editing. However, I think it's vital that they always be left open for improvement, especially as more scientific advancements come along over the next generation here in this state of Maine, I would love to see the potential for more

14:12:57 clinical applications of gene editing and more diseases being able to be treated or prevented. Cf.

14:13:05 Has been anything but easy, and has shaped my life and unimaginable ways.

14:13:09 But what I can tell you, is that seef has given me a perspective on life that has been invaluable due to medical advancements and new treatments. I'm.

14:13:18 The healthiest that I have been in years i'm now a college student, and I played on attending law school.

14:13:23 The current estimated life expectancy is someone with Cf.

14:13:28 Is in their mid forties. I use this information and choose to live my life to the fullest extent possible.

14:13:33 I'm hopeful for the future and further advancements that I know will be made.

14:13:40 I'm both excited and hopeful to be able to discuss the prospect of genome editing and the potential it holds to treat, the and many other conditions Once again i'd like to thank you for the opportunity to highlight

14:13:53 Cia, and to be a part of such an important conversation.

14:14:00 Well, thank you for being on the panel and agreeing to participate.

14:14:03 And thank you for that. Helped to see behind the curtain of a diagnosis what it's really like to have the condition.

14:14:09 So thank you for your bravery. Yeah, of course, Thank you.

14:14:14 Any questions for Abigail. Yes, Representative Simon. Hi.

14:14:21 Thank you for being here and sharing your story we were talking before about risk. And so, if if you if there was a treatment out there that you could, you could receive how risky, what's your own feeling about risk and what would you be

14:14:38 willing to to try. Yeah, as a cf patient throughout my whole life.

14:14:46 I've been involved in a lot of clinical trials so I've already had the experience i'm assuming a lot of different risk.

14:14:53 Cf is a progressive disease, so unfortunately it does only get worse as you go on.

14:14:58 It can be managed, and, you know, treated to delay all of that.

14:15:04 But I think that if it were me I definitely would assume the risk.

14:15:07 I think that the benefits would outweigh the risk. I know for me personally I think that a lot of other people with Cf would probably say the same thing.

14:15:18 Thank you. Yeah, of course, any other questions or comment from folks remotely, or in the room.

14:15:31 I'm not seeing any way so you can revert Oh, yes, Robertson dagger.

14:15:35 Hi I just wanted to not expressed appreciation for also adding to what it's already been said, we get tremendous insight and and benefit from from hearing and I know that you've done you've been in

14:15:51 a ambassador of sorts for in many forums, and so really appreciate you bringing yourself, and and your story here.

14:16:01 Thank you. have a gale. Were you one of the people advocating for the rare disease registry?

14:16:07 That was passed in the last session or so.

14:16:10 Yes, I was a part of that yeah that's what I that's what I said.

14:16:14 I remembered. Okay, so that's our modest small dip bar to in the ocean effort at least for now cataloging some of the rare diseases, and we've added cystic fibrosis system others to our

14:16:28 registry that'll eventually get hooked up to a national registry, and what was the other one?

14:16:35 It was just added recently, huntington's korea was added to the list, too.

14:16:42 So thank you for your efforts in that regard it worked I don't see any other questions.

14:16:50 So you can, revert to being a panelist member no longer a presenter.

14:16:58 So representative, the honorable Tina riley it's your turn.

14:17:05 Why, thank you. I appreciate the the opportunity to share my story.

14:17:10 My son Brian age 22 has dishon muscular dystrophy, which i'll refer to as Dmd.

14:17:16 It's the result of a mutation in a gene on the X.

14:17:19 Chromosome brian, is missing a approximately 79 base pairs of the 2.2 million that make up the distrophen gene because it's Xlink. It occurs almost exclusively in

14:17:30 boys is fatal in his face, inherited diseases go this one's relatively common distrophin is a protein found in different forms and tissues, including the retina and the brain, but especially in muscle.

14:17:43 tissue. The lack of dystrophen causes muscle tissue to die off, increasingly replaced by useless adipose tissue.

14:17:51 About a third of those with Dmd. or cognitively impaired, and cognition is affected to some degree in most childhood with Dmd.

14:18:00 Is marked by frequent falls, which often cause broken bones and other injuries.

14:18:06 The heart, being a muscle, is affected, as are the muscles responsible for breathing.

14:18:11 As the body weakens those functions fail without intervention to Shan patients typically dying. Their late teams or early twenties advances in supportive therapies, have allowed some people with descend to live into their forties and

14:18:26 fifties early fifties, but not because the disease process has been slowed, but because we've learned so much about supporting patients who live with respiratory and cardiac failure i'm gonna tell you of my experience as a parent of

14:18:41 a child with a single gene disorder. Not every single gene.

14:18:45 Disorder causes. The loss of physical function. as we've just seen is that we deal with in Dashen and others.

14:18:52 Experience of this disease varies widely. all i'm bringing to this table is my lived experience, which I intend to share to an extent that I generally avoid.

14:18:59 This is going to be tough i'm gonna talk about the physical cycleological, financial and social impacts of a cruel disease.

14:19:07 Unless you're a cold and heartless person you're gonna feel awful about it.

14:19:11 But it's our job to set aside that natural reaction to focus on how different everyday life is for those impacted by a disease of this severity.

14:19:21 I'm reading this to you to make sure I cover all my points without rambling, and if I need to pause, I hope you'll bear with me.

14:19:28 I was blissfully ignorant of dishen until Brian was diagnosed shortly before he turned to, which is younger than most.

14:19:36 Spring break of third grade was his first unplanned hospitalization for pneumonia.

14:19:39 When he was 12 he stopped walking. Care needs expanded to include repositioning every couple of hours each night, and soon I was exhausted to focus my energy on caring for him.

14:19:52 I left my job as an E, and I tech at the paper mail, abandoning my career and cutting our household income in half, because muscle strength is not perfectly symmetrical, as the trunk muscle

14:20:05 weekends, trunk muscles, weekend scoliosis often occurs, and it tends to worry, worsened rapidly.

14:20:12 Once the Dmd. or stops walking, the deformity can become grotesque, causing pain and difficulty breathing or even sitting.

14:20:19 When Brian was in middle school. Like many others with the Md.

14:20:24 He had 2 metal rods placed along the length of his spine and into his pelvis, permanently fusing them well.

14:20:30 The surgery went well. the aftermath did not and he and I spent months housebound and exhausted.

14:20:35 I didn't get more than 15 min away from him for well over a year, but he recovered, and he was able to keep up with his classes. thanks to the extraordinary efforts of several key people at school.

14:20:48 He graduated in 2,018. in the top 10 of his class.

14:20:52 He played the saxophone in band. He was active in the National Honor Society and robotics.

14:20:58 One is school's, geography being competed at the national level is the forestry expert on the main state championship.

14:21:04 Environmental and virus on team. He is often asked, sometimes in a loud and carefully enunciated voice, Did you go to regular school, and I wonder what people see when they look at my kind, brilliant, and witty son families

14:21:21 like mine, struggle with a reduced income, and additional costs.

14:21:26 Accessible vehicles tend to be monstrously expensive.

14:21:28 They get terrible gas mileage, and the hourly rate of the one certified mechanic within striking distance is more than twice what my regular mechanic charges we often wrestle with, whether or not Oh, my to spend money on

14:21:43 adaptations that may help, because the constant loss of function going to re render accessibility.

14:21:52 Features moved at some point in the future, finding or making adaptive features that will allow Brian to live his best.

14:21:58 Life is difficult and often expensive, and there are no meaningful guides to doing it right. while those choices do sometimes turn out for the best.

14:22:07 They invite a lot of second guessing and painful regrets.

14:22:10 Government assistance programs are a vital lifeline, but the process for getting and keeping benefits is intrusive and often nonsensical in ways that often leave me angry, humiliated, and worn out.

14:22:22 Fatigue is a hallmark of Dmd.

14:22:25 Partly because the body has to work so hard to do anything, and partly because the absence of distrophin in the brain causes a buildup of the products of metabolism in the Brian cannot lift his hands or roll

14:22:38 over in bed. It takes an hour to get him dressed out of bed and ready for his day.

14:22:44 Showering has recently become a 2 person. Assist his bowels are the only arbiter of our schedule.

14:22:49 They've kept us under siege for days at a time and sent us to the er on several occasions going anywhere.

14:22:57 Unfamiliar is a nerve is nerve wracking, because accessibility is never a given, and often Brian prefers to stay home rather than deal with the inconveniences that we run into in the outtime in

14:23:08 the outside world watching him miss out on so much, just breaks my heart.

14:23:14 At 22 my son needs machines to get out of bed to cough, to move about he's vulnerable.

14:23:21 A minor car accident, or the common cold, or threats to his life.

14:23:27 When the pandemic hit we locked down completely.

14:23:29 No one came into our how home for months pry has a ventilator that he doesn't use as much as he should, and a device that allows him to feed himself.

14:23:40 But it's not going to be enough i'm sorry probably has a ventilator that he doesn't use as much as he should, and he just got a gastrostomy tube that we hope will stop his

14:23:51 ongoing weight loss. Eventually the arm support device that allows him to feed himself is not going to be enough.

14:23:57 And I wonder whether we'll end up hand feeding him for some time before he loses the ability to eat altogether.

14:24:04 I dearly wish she would get comfortable with non-invasive ventilation, and I wonder if he'll choose a trachy Austin, when his breathing muscles can't get the job.

14:24:13 Done. Fortunately he still has enough hand function to use a computer and cell phone, but losing that is going to be a game changer for the whole family.

14:24:22 The Greek that I carry is difficult to categorize.

14:24:26 The accumulated and impending losses are heavy.

14:24:30 Caregiving often requires that I be the instrument of my son's humiliation in ways that could have been scripted by Hitchcock or Stephen King I don't know which I feel more

14:24:39 losing him or outliving, them. i'm not depressed. but in 20 years but 20 years of cyclical grief, frequent crises have left the mark, and there's really not a lot of help for it on the flip side I

14:24:53 have the rear good fortune to have a wonderful adult son, who is a daily joy to live with.

14:25:00 Some of my fellow parents seemed to hold up as well or better than I am, but others do not.

14:25:05 Family members sometimes retreat into shocking states of denial, a phenomenon that this panel should contemplate.

14:25:13 Children with the md are not served by attempts to strengthen muscles, and I've seen them hurt by parents who can't accept the reality of their children's limitations i've seen parents spend thousands on

14:25:24 sketchy treatments in other countries. They often come home raving about their son's immediate improvement, but they go sign as it becomes clear that they wasted their money.

14:25:33 It's good to believe that a cure may come but the reality that we simply don't have one yet is devastating, and it's too devastating for some people to come to terms, with since Brian was

14:25:45 diagnosed in 2,002, and I first learned of the emerging research on molecular medicine.

14:25:51 I felt sure that one day there would be a meaningful treatment for dishen, and that day is finally beginning to dawn with the first molecular treatments becoming available.

14:26:00 There are already treatments for some specific to shen mutations, and there are a couple in the pipeline that would address the deletion that caused my son's illness. as expected the price tag is out of reach for most

14:26:12 private citizens, at least for the foreseeable future.

14:26:16 Finding a balance between providing those costly treatments and stiffguarding public dollars is a call order.

14:26:23 Right now. drug utilization reviews set limits to feel arbitrary.

14:26:28 A child with the shen may be deemed ineligible for an expensive medication.

14:26:33 After losing the the ability to walk, after losing the ability to walk, or after reaching a certain age.

14:26:41 To me that feels short-sighted and ableist.

14:26:44 But the ethics of access to Life-saving life, saving treatment are never easy.

14:26:48 We should explore the question of whether the scientific development at hand is different enough from previous breakthroughs.

14:26:58 That our approach to equitable access should change

14:27:03 Thank you. ,

14:27:10 Another look behind a diagnosis personal Any questions of Tina before we cool

14:27:25 Representative. Zig and I were quite intentional about wanting to make sure that, people had a chance to hear.

14:27:32 Oh, and see if you in detail. But what some of the impact is i'm just kind of these kind of conditions.

14:27:38 So thanks for being on the panel thank you and i'm Sorry to be such a downer.

14:27:49 Really no way around it. Sometimes reality is hard

14:27:55 All right. I'm not seeing any other questions so Now we can hear from Benjamin King Logan Docker

14:28:07 Right. So good afternoon, Senator Claxton, Representative Zeiger, and the other honorable members of the advisory Panel.

14:28:15 I greatly appreciate the opportunity to speak with you this afternoon, and participating in this way.

14:28:22 I've been here all day it's it's quite enlightening to hear about the experiences that we just heard.

14:28:32 And certainly appreciate this this great work that the committee here is trying to accomplish.

14:28:40 My background is in genomics, in bioinformatics, and I've spent my career witnessing this tremendous revolution in our ability to sequence Dna.

14:28:55 And of course the this is something that we learned a lot from from from Dana.

14:29:00 This morning, and I I use those technologies every day in my research to characterize genomes.

14:29:09 Of individuals. but also in other research I characterize patterns of gene expression.

14:29:16 So how those genes are turned on or turned off in response to different pathogens, including the flu.

14:29:24 So influenza virus. and these technologies are are really amazing, and that they allow us to understand, eventually, by doing further testing the mechanisms of the human disease.

14:29:41 And I remember the the first large genome project I worked on was characterizing 1 point: 6 million base pairs of the gastric pathogen.

14:29:51 He look back to Pylori and was was really exciting.

14:29:54 But now that that see like a high school project and and that you cannot buy

14:30:02 You might want to look out this fall a company called dante labs offers Black Friday specials. and you can have your, you know, human genome sequenced for $149.

14:30:16 And And so this is something that's that's Very accurate, and that they're basically sequencing all roughly 3 billion bases in your genome 30 times over.

14:30:26 So It's highly accurate and But I I must say you don't have any financial interest in don't say life, but but it is something that that really makes genomics, something that is going to impact

14:30:40 all of our lives just as We We talked about this morning about some of the the applications of this technology to look at ancestry, but also even with prescribing different different drugs.

14:30:55 One course that we offer at the University of maine we train undergraduates in some of these techniques to actually look for mutations in a gene that's called sip 2 c.

14:31:07 19, and and that gene if if an individual has one what we call a leo, one version of that gene they cannot metabolize many common drugs like plavics whereas if they have the other allele they're, fine

14:31:21 and so they're they're learning about this this field of personalized medicine, and and certainly we'll have great benefits ongoing, and to talk a little bit about going somewhat off script some of my research that

14:31:40 pertains to to gene editing gene editing. and it is is something that, as we use different animal models to understand the mechanisms of human disease, that tool, whether it be crispr or some other type of gene

14:31:57 editing tool is essential to this this research, so we can deliberately engineer. in my case, zebra, fish, zebra, fish are a common aquarium fish, and there are a number of researchers at the

14:32:11 University of Maine that that study this and one unique characteristic in that.

14:32:15 During the first few days of development they're transparent and we can engineers zipper fish where certain cells fluoresce different colors.

14:32:28 In my case we're using different zebra fish where the neutrophils one type of of immune cell close green, whereas macrophages will be read, and then I have in my laboratory different kind colored

14:32:43 influenza viruses. So they've been engineered to express different fluorescent proteins, and so we can use high resolution confocal imaging to actually watch the neutrophils and macrophages

14:32:57 traffic to the site of infection. and actually start to clear the virus, and it's something that you can't do in a laboratory mouse.

14:33:06 Certainly you know it. it's something where the zebra fish is a unique resource to use. And so we have to have these ways of of engineering. the genome of the zebra fish to fluoresce

14:33:24 these to express these different fluorescent proteins in the specific cells that that we want.

14:33:30 And likewise there. There are many applications where if you're trying to study a particular disease.

14:33:35 You can do what's called a genetic screen where you can systematically.

14:33:42 Engineer mutations individual mutations, and essentially every gene in the zebra fish or whatever cell you're, you're interested in studying and actually measure Then the response.

14:33:54 And and know that a certain set of genes are actually required for some important function, and and the ability to to make these sort of precision edits to to the genome is is something that's that's incredible So

14:34:13 certainly. Maine has a really strong history in in biomedical research, and we'll hear from a colleague of mine. Dr.

14:34:23 Laura Reinholt, next from the Jackson lab.

14:34:27 They're they're just so many different applications and really allow us to better understand how Genes function, and and hopefully will also allow us to then engineer new therapies as as we go there are 2 specific

14:34:48 suggestions that that I have for the panel to consider, and I think they've been touched on to some extent.

14:34:57 But I you certainly want to try to elevate them.

14:35:00 And the first Well, they both have a common theme. where, we need to have main as a larger community of of residents and and to have productive dialogues on human gene editing and These technologies.

14:35:19 Exist. they are being used and I would say in the future.

14:35:25 There will be other ways that they're being used that that people will not approve of, and it's it's something where, if we could put position the State by one increasing investments in public pre K.

14:35:42 Through 12 and postgraduate education and research in health and biology that would help prepare residents to contribute to these ongoing dialogues on on human gene editing.

14:35:56 It would increase their awareness of genetics and related technologies.

14:36:01 And then expand the number in diversity of individuals who could participate in those discussions within main

14:36:09 Similar investments that, you know, are somewhat similar to this are things like the main economic Improvement Fund. me.

14:36:18 If that's done a fantastic job and has transformed my own research program.

14:36:23 But it it's something where so strategic investments can pay off and

14:36:31 You know, as an example, I was looking back to a photo on my my shelf in my office, and a team of 5 students that I had 4 years ago.

14:36:42 4 of them are in PHD. candidates. one at Dartmouth,

14:36:48 2 at University of Rochester, and 1 18 years Percy of Maine, and then the fifth is now

14:36:53 Just entering her first year at Harvard Medical School.

14:36:57 And providing students really meaningful. And research experiences can pay off, and hopefully they will eventually come back to Maine as well in the future.

14:37:09 But having these investments, I think, can broaden participation in these discussions in the future.

14:37:18 The second idea is something that yeah, actually fine.

14:37:27 Tina Riley had spoken about in that it would be nice to support communities of patients and their families who have these different diseases.

14:37:40 A broad spectrum of diseases she had mentioned having an email list right so to network with with patients and families and to support these communities of individuals is is something that I think can also facilitate the dialogue on human

14:37:57 gene editing, and there were great organizations out there like the Cystic Fibrosis Foundation, and and others that not only provide information for patients and their families, but it also they work as advocates they actually this the

14:38:17 cystic fibers. this foundation is actually funded.

14:38:21 The development of some therapies it's it's really an exemplary organization, as an example.

14:38:28 There's also the national organization for rare disorders. that that has over 300 patient organizations, and and many of these are single gene disorders and and that's quite useful.

14:38:40 And I I recognize that this spring the main rare disease, advisory cancel, was was passed, and and those are fantastic things.

14:38:48 But one could also think about, maybe expanding that to individuals with more complex diseases like alzheimer's or diabetes.

14:38:58 Chronic kidney disease, so that there are communities where they can share information about gene therapies.

14:39:05 That they can also have dialogues about

14:39:08 Even Germline gene editing, as as those things will certainly come up in the future.

14:39:15 And and so I think that having sort of this too prong approach to help raise awareness of gene editing and the related technologies would would help broaden and expand the inclusion of individuals in the in the diversity

14:39:33 of viewpoints that are all contributing to the ongoing dialogue and as sort of a long-term investment.

14:39:42 I think also trying to expand investment in clinical and translational research, so that there are more clinical trials that are maybe even led by in researchers in Maine.

14:39:59 But also expanding. access. Certainly many clinical trials require visits to a specific hospital and traveling down from Prescott is is really not something that's that's practical for many people to to say get to

14:40:14 Boston on a regular basis. So yeah, just as with the would like to say that.

14:40:25 As we discussed, the ethical, legal, and social implications of gene editing are certainly complex and interdisciplinary.

14:40:33 And certainly the wanna speak on behalf of Maine's Public Research University that that we certainly support the work of this panel, and and certainly view it as as important work.

14:40:49 So. thank you very much. i'd be happy to answer questions thank you.

14:40:56 I'd forgotten about how useful zebra fish could be and now they're a lot more useful than I thought they were right.

14:41:05 Yeah, every You know, there are a variety of of model organisms.

14:41:09 We call them, whether it be the fruit fly. Dana had mentioned that. what we call the round rooms.

14:41:18 See elegance. they all have really important. yeah.

14:41:26 I was just thinking see if anybody on chat had a question.

14:41:31 Thank you very much, Dr. King. you mentioned 5 that you're those 5 students and yes, they're they're at yeah.

14:41:40 Another phase of their education training What do you think it would take for several of them to want to come back to Maine? Yeah.

14:41:50 So I I know that So to begin with, 4 of the 5 grew up in Maine and and I I know that they all would like to stay in in Maine.

14:42:05 It is something where you know, there there are a finite number of of job opportunities.

14:42:13 Certainly there. There have been lots of growth in in some of the sectors like in the Portland area, with some biotech, and there's Aidx and and other things I personally worked at the Jackson laboratory for 10

14:42:28 years. You know that's a great place. but if if we did make more investments to grow.

14:42:39 Some of the Research enterprise, the biomedical Research enterprise in Maine.

14:42:43 Then I think there would just be more opportunities if you look at the growth of some of the institutions.

14:42:52 That that even at the University of Maine or or elsewhere.

14:42:57 You know they're they've been very successful in getting Federal funding to expand their research, and in some cases have been spin off companies, and and that sort of thing.

14:43:09 So I think it does take a long time. but to provide those opportunities to attract and retain the students.

14:43:18 They do require long term investments in in being designated this past year as an R.

14:43:22 One research, Great really okay. One of the things that that came to light during the hearings in the in the Committee on this bill was the Millikan Foundation, the Middleican Institute which apparently publishes

14:43:42 report on they're they call the signs and tech index, you know.

14:43:48 Each States it's a measure of how each much each state invests in science and tech as a sector of the economy.

14:43:55 And it found that maintenance Maine had dropped 5 spots was ranked forty-third in 2,020.

14:44:02 I think the the next, I think, comes at every 2 years, so we might get another report soon.

14:44:07 But it it would seem like we have room to grow great.

14:44:12 Great is that what you're seeing from from and There are some programs that main participants in one is called the the main inbre program.

14:44:23 That that is headed by not just that island biological laboratory.

14:44:27 And I certainly is the co-director for the bioinformatics square of that Grant that provides funding for that course.

14:44:34 I was describing where the students are actually genius typing, we call it sip, 2, c.

14:44:41 19 and and learning about personalized medicine the and you know they they also support a number of other courses in the State.

14:44:51 And you know that that's a really good example of how that investment, and they have lots of statistics of you know how many dollars could be traced back to to that Grant.

14:45:06 That is is one example there are also other nh programs when it's called a cobri and that's a grant mechanism that's both transformed the main help the institute. for research they've had a

14:45:24 number of these grants and provide so like they recently renamed

14:45:31 Main Medical Research Institute, and main help institute for research and you know that's where these investments in this case from the Federal side.

14:45:39 And these are competitive grants you know it's it's where it's provided valuable funding to recruit new factory and retain them to build building those those kinds of things and

14:45:53 It's also been important in the growth of mount desert island biological laboratory.

14:45:59 So yeah, there there are those things And but you know, state funding is is obviously something that.

14:46:10 Yeah, okay. if we were able to expand that institute ways that would be absolutely transformative.

14:46:16 And that, I imagine, is also private funnels that you know It's not just the States that are investing in in there's like Rue Institute is you know in but there's so

14:46:32 opportunities for industry to Wanna: innovate here. Yeah.

14:46:39 Okay, thank you very much. you're welcome Yes, please

14:46:50 So you alluded to. There are some things that people really are sure they want.

14:46:56 And then there's some more controversial things sort of brewing in your mind around when we when we get to gene editing in different sectors.

14:47:03 I wonder, are you ready to talk with us about those, or do you want?

14:47:08 Or is that for another time? Because I have I have a few in my mind, too?

14:47:12 I'm curious to hear what where you see some of the controversy.

14:47:18 Yeah. So with regards to somatic gene editing you know, I think there is great promise.

14:47:27 So that's where typically there's maybe you know one organ, or you know, in the case of treating treating a disease.

14:47:38 The the sma drug or or or the like or treatment rather and and those, I think, beyond some of the safety concerns and and things that that is something where I think there'll be a lot of growth, and but with

14:47:57 regards to yeah editing germ cells that that's very, very difficult.

14:48:05 The but what makes it even worse is is that

14:48:12 You know, I think there will be people that are just going to go out of bounds.

14:48:18 And, you know, ignore whatever regulation there is, and and end up successfully Editing?

14:48:28 Human genomes. And yeah, that that just yeah is is very, very difficult.

14:48:37 But with any type of treatment or study there's always the risk and benefits, you know, if you have like I have a a study that I I do in collaboration with the Northern Light Eastern main medical Center.

14:48:53 And you know the Institutional Review Board to explain all of these things.

14:48:57 But yeah, I I It it's it's something where you again.

14:49:06 People are going to decide whether that study should go forward. So who who are the people that are actually deciding that?

14:49:14 And if that study ends up editing this German line then you know that decision is gonna permanently alter human evolution.

14:49:25 Potentially. Excuse me. Yes, thank you for being here.

14:49:31 Can you do know enough about the irv process. in a hospital system to talk about it a little bit, because I think it'd be good for people to understand that I talked a little bit I don't serve on the the irb and

14:49:44 certainly there. yeah. other Some of my faculty colleagues are specialized in bioethics and and the like.

14:49:52 So but yeah institutional review boards are a a committee that oversees research.

14:50:06 That's being done at an institution and and So they are you when you apply if you have a protocol for a study that you want to conduct with with patients It's a very lengthy application basically that.

14:50:26 you have to describe. You know what the objectives of the study are.

14:50:32 Your protocol the risks and the benefits.

14:50:38 You have to give example text of the informed consent form.

14:50:42 You know what are the all of the data you're going to collect in in those things, and then that's evaluated by a committee.

14:50:50 Of physicians. but also there's typically a number of the clergy and other members of the community that that participate in that organization.

14:51:06 Right, that committee, and and they're going to approve or or ask for revisions, or just you know flat out.

14:51:14 Refuse for that that study. to go forward and then periodically there's review to make sure that you're complying to those that protocol.

14:51:25 So yeah, is that what kind of what You're thinking there's also often participation for hospital council.

14:51:32 Hmm. when it gets close to it being less so there are legal people, social hospital. So you know that's an unnecessary risk for the organization.

14:51:41 So support that getting the surgery so that's another that kind of procedure that's another reason, though little layer of a review.

14:51:50 Yeah different kind. Dr. Chessa. yeah just kind of full disclosure.

14:51:59 I'm a kind of long term member of the health irb. I think I've been on the committee for 15 years now.

14:52:07 So, and then through it, quite a bit if folks have more detailed questions.

14:52:12 I'm happy to service a resource for that so I I should also full disclosure.

14:52:18 You mentioned one of the main health covers i'm a I'm, one of the leads on the main health acute care.

14:52:24 Cobra. Thank you. We tried to make sure we had lots of different resources available on the committee, and we only have 4 meetings. all right.

14:52:42 Any other questions. comments. little CD: So thank you for being here.

14:52:47 Thank you. So next will we joined or have already been joined.

14:52:55 We'll turn the icon for or reinholes welcome, thank you.

14:52:58 Good afternoon. so my name is laura Reinholt and I'm. an associate professor of genetics at the Jackson Laboratory, and i'd like to thank Senator Claxton Representative Zager

14:53:10 and all the members of this advisory panel for inviting me to speak today.

14:53:15 So in this presentation I was asked to answer the following two-part question, which is, What should the state of man do regarding gene editing within my field of expertise to best benefit mayors in the next 5 years and

14:53:28 subsequently over the next generation. And answering these questions, I offer my perspective on what I think is the positive transformational impact of gene at any technology in biomedical and clinical research.

14:53:39 Broadly and more locally. also here in Maine, I have spent the majority of my scientific career here, in Maine, having moved here for a post-doctoral fellowship, opportunity in 2,000, and one I became a scientist in the

14:53:51 first place, because I was fascinated by genetics and I knew I wanted to be as close to that work as possible, and I really didn't dream that I would be able to do that here.

14:53:58 In the State of Maine and i'm incredibly grateful to the mentors that helped get me here, some of whom are native men who are able to return to establish their careers here in addition to my personal background my

14:54:11 presentation also reflects the collective experience of my colleagues at the Jackson Laboratory. A nonprofit institution at the forefront of biomedical research here in Maine laboratory holds over

14:54:22 a 100 active grants from the National Institutes of Health, and employs over 1,800 people across 3 locations, including the headquarters.

14:54:29 Here in Bar Harbor, the main Cancer Genomics initiative in Augusta and in an innovative production.

14:54:35 Facility in Ellsworth, which was actually constructed with matching competitive funding from the Main Technology Institute.

14:54:43 The lab invests substantial capital every year in research tools used by scientists at each of these locations. in our mission to discover precise genomic solutions for human disease.

14:54:53 This work is vital to enhancing our quality of life through better health and high quality jobs, life.

14:54:58 Scientists research and development is essential to maine's innovation economy and is highlighted in numerous recent reports, including the main economic Development strategy.

14:55:04 10 Year Plan reports from the Main Development Foundation, main State Chamber of Commerce and educate Maine.

14:55:12 Main ability to sustain and grow Its innovation. economy is related to our continued access to critical technology, including Gene editing the focus of today's discussions during my own career.

14:55:23 I've experienced 2 technological inflection points. These were next generation, genome, sequencing technologies, and then followed by Crispr castine, based gene, editing technologies.

14:55:37 As Ben described next generation, sequencing technologies allowed us to move from sequencing a single human genome for 2.7 billion dollars in 13 years to sequencing a single genome and under a day for $1,000 and even less

14:55:50 as it turns out, if it's black friday, we call these kinds of technologies disruptive because they open up completely new industries and fields of research, and that has certainly been true for genome sequencing the results of these efforts are

14:56:03 hundreds and thousands of genomes that have revealed incredible genetic variation across the human population.

14:56:11 The form of molecular differences in the Dna sequences that make up our genomes. and I refer to these as genetic variants.

14:56:18 As scientists. We could now begin to ask which of these variance cost disease?

14:56:23 Which of them make us susceptible, resistant to certain environmental exposures, Infections?

14:56:27 Which of them determine if a drug will work for some or not others.

14:56:31 But we knew we would need significant innovation in genetic engineering technologies to begin to tackle these important questions.

14:56:39 Crispr past 9. based gene at any technologies, are now the enabling technologies that are allowing us to answer these questions combined with sequencing technologies, we can identify what we will engineer and with crispr past

14:56:52 9 and related gene editing technologies, we can finally accomplish that engineering at scale.

14:56:58 Considering these watershed advances, my peers in the scientific community also quickly recognized the potential societal impact of easy, genetic engineering.

14:57:06 If it were to be applied to the germ line which We've mentioned several times today, and I just wanted to readerate that scientific organizations like the National Academy of Medicine, the National Academy of Sciences, National

14:57:18 Institutes of Health and the World Health Organization have all articulated a moratorium on gene on germ line editing. And so it was.

14:57:27 In fact, the inventors and the users of the technology who were the first to self-regulate many years ago.

14:57:32 And it was later that government regulations followed soup. for example, the Us.

14:57:37 Food and drug administration will not approve a gene therapy where there's risk of germline editing.

14:57:42 So with oversight from within the scientific community and at the Federal level, Gene editing is now a state of the art tool.

14:57:50 That is used extensively in biomedical research, and I think Ben provided a nice specific example of that in his own research.

14:57:56 I'd like to offer 3 examples of how myself. and my colleagues at the Jackson laboratory use this technology in our labs, and this covers 3 main areas, one being discovery. the next is disease modeling in the

14:58:11 third is the development of therapies. So in discovery, essentially high throughput gene editing in cell lines and in simple model organisms like mice, allow us to ascertain the function of the millions of genetic variants that have

14:58:25 been discovered by genome sequencing projects. This simply could not be done efficiently prior to gene editing technologies.

14:58:33 In this application Gene editing allows us to identify the most important impactful genetic variance by changing them and then studying the resulting physiological consequences in cells or in model organisms in disease. model and we as it we edit

14:58:48 the genomes of laboratory, mice, or other model organisms, to introduce the specific genetic variants that cause diseases in people.

14:58:56 And this gives us experimental systems where we can test interventions and models that carry the same disease, causing mutations as people.

14:59:04 This is one application actually, where germline engineering is desirable and an animal in an animal model creation of these models is what we mean when we just, when we, when we use the term precision modeling at jacks, we

14:59:16 have a center and Nih funded center for precision.

14:59:18 Genetics as well as a rare disease transitional center, both of which are focused on building these important disease models.

14:59:25 And I wanted to highlight that one of the important models created by Jack's was actually the sma model that was used for the development of spin Rosa, and in fact, over the last 5 years the number of Gene

14:59:39 edited, based to these models that have been created and and shared with the scientific community by Jack's, has grown by over 2 orders of magnitude.

14:59:47 So, just 5 years ago we had perhaps a couple of disease models that were generated using genome editing technologies.

14:59:55 Now we have several 100. Finally, the use of those models in the development of pre clinical therapies.

15:00:03 So in this application gene editing of the somatic cells and tissues of a disease model, can be used to correct the disease, cause invariant, or replace the affected gene product.

15:00:15 The laboratory mouse and genetically engineered mouse models are critical in this pre clinical research, because, like humans, their mammals.

15:00:21 But we can manipulate their genomes. We can control their genetics as well as their environment.

15:00:25 At Jacks we have an nih funded somatic cell gene editing center that's completely focused on advancing these methods of gene editing. and, of course, the next up in this process is to take the knowledge that

15:00:37 we learn in the animal models, and apply that knowledge directly.

15:00:44 To investigational new drug applications and clinical trials and human patients.

15:00:50 So these are examples, Gene editing being used to identify, which variance, protector cause disease to engineer human disease, causing variance and model organisms for preclinical development of new therapies.

15:01:00 These are all happening at the Jackson Laboratory, where, as I mentioned, Gene, editing technology is helping us advance our mission, discover the precise genomic solutions for human disease.

15:01:11 So coming back to the original question of what should the state of man do in the next 5 years, and subsequently subsequently, over the next generation?

15:01:19 I i'd like to concur with dr and kings presentation.

15:01:24 The State of Maine should promote awareness and education in life.

15:01:28 Sciences in both schools and community organizations to build on Ben's recommendations.

15:01:33 One way the State could do more is to invest in K.

15:01:36 Through 12 education. by supporting organizations and programs that are already working to support teachers in schools.

15:01:44 And this usually brings Federal dollars into the State to do so.

15:01:47 Existing programs, such as the Jackson Laboratories teaching the genome generation has reached over 9,000 students at 60.

15:01:53 One high schools in Maine the personal genetics, education programs, faith, partnerships with which engages with faith communities on how we make collective decisions about how and when and whether to proceed with human gene editing and this can be expanded

15:02:06 to reach more students, more schools and more community organizations in the State. In my view, most main citizens are not well informed about biomedical technology, nor have adequate resources to learn more or engage in conversations about

15:02:19 biomedical research, let alone specific technologies like gene editing in the near term.

15:02:25 This advisory committee should be confident in recommending policy that enhances education and awareness.

15:02:30 Knowing that stringent Federal regulations, limiting the use of gene editing technologies already exist.

15:02:35 Also in the short term I suggest the State build and sustain an environment where discussions and panels like this become the norm and not the exception.

15:02:45 For example, my colleagues in Irs excited to see that Maine will establish a rare disease.

15:02:49 Advisory Council to discuss and help solve issues that impact patients and caregivers of people with rare disease.

15:02:55 I think we'll see an intersection of gene editing technology discussed in the context of this panel with the interest of the rare disease community who are in, who are in a position to benefit most from these technologies in the

15:03:05 near term, finally over the long term. you know I. The The idea of promoting equity and in medical care is is quite important.

15:03:16 Main show, invest in medical research, talent, and infrastructure, such that patients are not prevented from accessing the future genomic treatments due to lack of proximity to major research.

15:03:26 Hubs like Boston and Cambridge. I suggest to Maine.

15:03:29 Make it a state priority to bring these medical centers closer to patients find investing in an environment where medical research can be performed and clinical trials can be delivered closer to home.

15:03:39 I thank you again for the opportunity to present and also for your service, and for bringing this important conversation into the public sphere.

15:03:46 And i'm happy to answer any questions

15:03:59 Hi! Thanks for being here. Just a quick question. Are those knockout mice?

15:04:06 They, when you change the function of the change you take out is that what they're called

15:04:15 Sometimes they're knockout my actually change the gene product So when we're applying gene editing, I would say that in many cases we're going after making a very specific change in the dna when we're going

15:04:30 after making a knockout our goal is to basically remove the the functional regions of that gene, so that there's no gene product at all.

15:04:41 And sometimes those kinds of mutations do are are involved in human disease.

15:04:46 But other times It's more specific changes. in the dna that lead to a disease. and I think you know we heard about this example of Duchess muscular just dystrophy in this case if that is

15:04:57 a deletion. in justrophen but in other cases. there's point mutations, and I would argue that knockouts are actually pretty easy to make advice. and have been for many years even before we had chris

15:05:11 per cast 9 base technologies. But now that we have gene editing technologies that actually makes it much easier for us to make specific face changes to the sequence of the dna that.

15:05:22 And so that's a much more sophisticated way of changing a gene, and we can match those directly with human patient mutations.

15:05:30 Thank you guys Who do you sell the mice, too?

15:05:38 So we are the all of the people who receive mice from the Jackson Laboratory.

15:05:45 Are by our biomedical research institutions those are academic institutions.

15:05:49 They're also pharmaceutical companies the you know the lab also has research partnerships with pharmaceutical companies where we actually do the mouse work here at the lab.

15:06:02 Rather than sending the mice out And I wanted to add, You know the Jackson laboratory has a research program, and then we also have

15:06:11 This bio resource. So the the resource of the genetically engineered mice that we share with the scientific community.

15:06:19 And my particular role at the lab. I run a research lab.

15:06:24 But I do create some mouse models in my lab that I then are.

15:06:28 I can share those with the scientific community by taking advantage of the side of the lab that does that work.

15:06:34 Are there people you would not sell, the lab the mice to well, we we don't sell mice to people who are not qualified by medical research institutes, or you know.

15:06:46 So we you you wouldn't be able to order you have to have specific certifications.

15:06:55 In order to receive mice from the Jackson laboratory.

15:06:58 So an institution has to have what's called an a lac surface certification which is a national certification that gives you permission to use laboratory animals in your institution.

15:07:10 And so it's very similar to to an irb actually

15:07:15 So, and you have your institution must have a committee that's comparable to an Iv.

15:07:20 We call it an animal care and use committee and most of the institutions that that received mice from us have a lack certification, and that committee

15:07:30 We actually don't just review mice to anyone who doesn't have that in place.

15:07:34 Thank you. Yes, Dr. Chessly, Dr. Chess is your hand up again.

15:07:41 Yes, if it if it's okay kind of 2 technical questions that I I probably won't ask very well.

15:07:52 But you know, one of the the sort of cautionary tales you always hear is about off target effects.

15:07:57 So essentially I'm gonna ask you to explain that concept but you know, put another way.

15:08:05 One question I have is when you're using crispr and You're trying to make a specific change in a genome, how often do you sort of fail to make the specific change in we're looking for and then the second question

15:08:17 is, even if you make the specific change you're looking for how often are there sort of unexpected down the road?

15:08:25 Consequences of that in the organism

15:08:31 To answer that question. So the the original Crispr cast 9 based technologies that we started to use in mice.

15:08:41 Were technologies where the Crispr cast 9 reagents would create a cut in the Dna, and then the repair of that cut in the Dna would would create the change that we were trying to make and

15:09:01 you know the the that original form of the technology was less efficient.

15:09:06 And I think a lot of people, maybe sort of have that in their when when they're reading and thinking about Chris per cast not.

15:09:13 And they're thinking about off target or unexpected events that really a lot of those were more common in the early generations of the technology, where sometimes those cuts in the Dna weren't repaired very

15:09:26 efficiently, and sometimes you'd just be left with a deletion. losing a few base pairs of the Dna rather than making the specific change that you're trying to make So the So So in mice.

15:09:40 You know we we often would see those deletions more often than we'd actually see the specific mutation that we wanted to make

15:09:46 But what our approaches have improved tremendously and actually just even the last 5 years, and we have variations now at Crispr past 9, like you may have heard of base editing.

15:10:00 For example, these are variations of Crispr cast 9, where, rather than a cut being made in the Dna, a specific change in the Dna is made, and that's made a huge difference in, and how efficiently we can make precise

15:10:10 mutations. we've also learned a Lot about the off target effects that you describe, and that is, you direct your Crispr, Kasma and Regents to a particular place in the Dna.

15:10:22 But there might be another place in the genome that has a similar sequence, and that might be enough for an off target mutation to happen.

15:10:29 And so what we've been able to do now is improve the technology to minimize those off target mutations.

15:10:38 And we can also screen mice for those off target mutations, because we know exactly where they could be.

15:10:44 And so we go into the genome when we look to see if there are off target mutations that are being made

15:10:51 So. So now with the most recent iteration of these technologies, it's actually quite efficient, and we are able to deal with the off target effects much better than we could just 5 years ago, and I I only see that

15:11:07 improving and the you know, one of the advantages that we have with laboratory mice is, if we do detect an off target mutation, we breathe that mouse and remove the off target mutation simply through breeding you know

15:11:20 This is not a concern for creation of disease. models but it's definitely a concern for the clinical application of Crispr class, not based gene editing in people.

15:11:32 So a lot of the you know, a lot of the work that's been done with the base editors has been, you know, done with this focus in mind is of we need to make sure that the changes that we make in the Dna in

15:11:42 patients and somatic cells are very precise and that we are confident that we're not introducing off target mutations in people.

15:11:51 So I didn't give you numbers in terms of efficiency. But I would have to probably share a whole report with you on that, depending on exactly what the technology is and how efficient it is in mice.

15:12:02 But I think in people we we can't tolerate but very much inefficiency, you know, and people for therapy.

15:12:08 It needs to be highly efficient. Yeah, no thank you for explaining that. Another thing I want to point out to is some of the gene editing therapies that we're hearing about involve actually taking stem cells and engineering

15:12:19 stem cells and putting those stem cells back into a patient.

15:12:24 And and the advantage of doing that is, you can screen those stem cells for any off target effects.

15:12:29 Before you put those cells back into a patient and that's you know some of the early approaches for sickle cell Were done that way.

15:12:39 And in other applications are done that way. So there is a way of.

15:12:42 Even if you do have off target, you can. You can look for them and get rid of them before those cells go back into a patient

15:12:51 Yes, sir. Professor Renold they first of all thank you so much for your patience today.

15:12:57 You're you're the cleanup hitter and but so appreciate your expertise, and being here, my question is regarding antibiotics.

15:13:08 And because in in some circles in the medical literature there's a question of what what are we gonna do when antibiotics no longer work there's already some significant organisms that have developed resistance to current

15:13:25 technologies. we're about a 100 years in in the age of antibiotics, and just it's possible that that is gonna you know that will find that the microbes will win out ultimately But that some people are saying

15:13:38 that Crispr engineer in general in general might be an entirely different approach to treating pathogens or microscopic pathogens.

15:13:49 I recognize that That's not your field per se but in conferences, and in talking to colleagues, are you seeing or hearing much discussion about Maine's work in what could be a a huge need

15:14:06 therapeutic need in the future. you could say no if if not, i'm unfortunately not in my circles.

15:14:18 But I you might want to add that to your list of you know experts that you can draw from for future meetings.

15:14:25 Because it's it's just not something that I I can speak on fair enough.

15:14:29 Thank you. Really appreciate your presentation useful to the irb equivalent for lab research.

15:14:40 What was that acronym Ala? yeah so a lack That's the American Association of Laboratory.

15:14:49 Sorry for the acronym is escaping me.

15:14:53 The American Association of Laboratory Animal accreditation.

15:14:57 I may have that. not quite right. but if you look up a lac.

15:15:00 You'll find it And so this is this is a governing body that basically oversees all research involving laboratory animals.

15:15:09 And so the Jackson Laboratory is an a-accertified research institution, and anyone who is receiving mice from us.

15:15:20 They need to have basically what we call an animal care and use committee, and they need to have you know, animal protocols equivalent to irb protocols in place before they can use they can Basically, receive

15:15:35 mice from us. and in the and actually the constitution of your of the local animal care and use committees that are in place at these institutions.

15:15:44 It's very similar to an irv so you have you know we have veterinarians.

15:15:49 Comparable to Irb, where you might have medical doctors.

15:15:53 We have members of the local community. We have clergy, and we have the scientists who are proposing to do the work.

15:15:59 And all of our protocols have to be approved by this committee.

15:16:04 In order for us to, you know, to use animals in our own research, and we require anyone receiving animals from us.

15:16:13 To have the same. Is there oversight from the folks at Nih.

15:16:20 That's different from those 2 no mh has the same requirements.

15:16:27 So, for example, if if I if I receive a grant from the National Institute Institutes of Health, and I have proposed to use laboratory mice in in my research, I have to have you know I have to have a lac certification.

15:16:42 I have to have you know an approved animal.

15:16:46 Use summary there has to I have to be performing my research at an institution that has this committee in order for the grant money to even be transferred over.

15:16:54 So I don't get the Grant if I don't have those things in place.

15:16:58 Thank thank you for for that i'm not familiar with the nih world.

15:17:03 I'm, more familiar with the Irb, world but that helps are there additional questions.

15:17:09 Yes, Tina, not so much a question I just a piece of commentary for people who aren't from the I Rb.

15:17:19 World of the nih world and who really don't know anything about science, this kind of science, and I fit into that category.

15:17:26 I know a whole lot about my son's disease I don't know a whole lot about anything outside of it that we're relating to, and I keep hearing about mutations and cutting out pieces of the gene and and I think that there's

15:17:39 a piece that gets kind of missed for those of us who don't have that background which is that my son's mutation is on the distrophen Gene.

15:17:48 But it's only a small piece of It and different different distin Patients will need different approaches.

15:17:57 They? will they will need personalized Medicines for each of these and the mutations are I mean the there's a There's a database from over in the Netherlands.

15:18:07 That's just staggeringly huge of all the different mutations that have been found, and some of those are like with my son.

15:18:13 He's missing a whole exxon which is a a group of base pairs that's a varying length.

15:18:20 Others are missing large pieces of the gene that go well beyond that there's a point.

15:18:27 Mutations where a single base pair is just the wrong letter, and it does all of that.

15:18:32 And there are places where there are actually replications where a chunk of genetic material is repeated where it shouldn't be, and it can also have the so just to make sure that people who aren't experts understand that that there's

15:18:45 not one thing that's going to cure the shen right if they, if they get the one that cures my my son's specific mutation, they hit about 7% of the of the shen patients worldwide Yeah, that's exactly right

15:19:12 you sounds like an expert to me. I think that's a great explanation, and I and I think that also you know, you might wonder, or 10 different most models of Sma and growing.

15:19:15 And it's because you know depending on the type of mutation.

15:19:20 There may be a different type of therapy that needs to be applied.

15:19:24 And and in each time we need you know a mouse to help us do that, at least in in my world.

15:19:31 That's what we try to accomplish and the work that you're doing touches on those 3 points.

15:19:38 You made early discovery of function for portions of the genome, and then some disease modeling work before you can get to the prefundable Exactly.

15:19:50 And and I also want to point out that there our undiagnosed rare diseases.

15:19:55 So. the human genome sequencing projects are working very hard to sequence patients with rare diseases, and there are patients with rare diseases who remain undiagnosed because we actually don't know all of the mutations

15:20:11 yet that cause even rare diseases and people never mind complex diseases where you know, a rare disease typically is caused by mutation in a single gene.

15:20:20 But complex diseases. Many of the diseases that we that that affect lots of people involve many genes working together. And that's an even more complicated problem, because, you know, we need to first figure out what those genes and variants

15:20:35 are, and then we need the models so that we can test therapies.

15:20:39 And so I made a comment that I thought that the rare disease community might be the community who stands to benefit most in the near term, and that is because you know we're learning so much about rare diseases so quickly because of

15:20:53 human, genome sequencing and because there we're focusing on single genes.

15:20:59 And I think you know in the future we'll apply that to more complex diseases as well.

15:21:05 Thank you for that. any other comments or questions.

15:21:11 Yes, so I was thinking about where the heck did I think of that?

15:21:19 Idea of the looking at the costs of these these drugs, and it is the rare disease panel, the panel.

15:21:28 And I know that because in Aa. and appropriations that was what I was in charge of when we were running the table looking at the bills left over, and I so I read it carefully.

15:21:38 And so I think it would. It would be great for us to see Ld.

15:21:41 9 72 which is now chapters law and you'll see It's a big bill, and they have a lot of things to do.

15:21:50 So they're gonna be busy year after year but part of It is establishing a comprehensive plan for the management of rare diseases.

15:22:02 Yada Yada public and private organization for potential sources of funding.

15:22:07 Oh, no, that's not the one I wanted to read you Well, anyway, if we could get that, and everyone would would understand what's in there, and part of their charge is to look at payment treatments so representative, hymens

15:22:23 and has helped a segue into the next and last portion of our day, which only needs to last as long as people want it to. Before we do that, though I would invite people to submit anything you put into the chat

15:22:37 function that would be helpful to all of us and to anybody else who's interested party.

15:22:44 See interested parties. I saw a link to the chains, for instance.

15:22:51 If you could. if you could share that with Janet, that would be appreciate it so that we can get it out there and make it available to anybody who wants to.

15:23:06 Wants to be educated. Also the reference to the Black Friday special.

15:23:14 If you want to email her the link will we'll share that too widely, We're not endorsing it.

15:23:19 Then there's no vested interest to to be declared here.

15:23:23 So before we wrap up we've had a couple of people in the audience who've been very we've been very present.

15:23:34 We hadn't planned to hearing from anybody but Do you have something you would like to chip in at all You do have a seat we had not planned for portions for public comment.

15:23:49 But you deserve points for hanging in there all day.

15:23:52 So here we go. Thanks, i'll be brief I just wanted to take the opportunity.

15:23:57 Since you had mentioned it earlier to highlight, and provide some additional information just some questions that were asked earlier.

15:24:04 So I'm Mike mcconnell and I'm, the director of community excuse me Government community relations also at the Jackson laboratory in Bar Harbor, and I wanted to just provide some some additional color on

15:24:17 the the point that's been made about the economic opportunity for life, sciences, research, and development in the State, and particularly questions that are posed about, you know, bring students back to the State for careers and just some some points of information

15:24:34 Jackson Laboratory employs, as Dr. Reynolds said, about 1,800 people in the State.

15:24:41 Right Now we have 108 open positions that's current is it?

15:24:46 5 min ago when I checked. Those are not only entry, level positions, their senior level positions and middle-level positions.

15:24:54 I I thought it was interesting to point out that the average age of our new hires in the past, in 2021 was 31 years old.

15:25:02 So there's a they're Abundant opportunities for people to stay in the State of Maine, and when we look at our hiring practices over the last 10 years.

15:25:13 The majority of those hires are from within the State.

15:25:19 So out of. let me see here out of the 233 new hires that were made in 2,021 31.

15:25:27 We're hired from outside the state of maine the rest were hired from inside the State, so just an example of the opportunity that does exist, and will continue to grow for main students and trainees who are brought up in the State to stay in

15:25:41 the State and contribute to our, you know, scientific mission.

15:25:49 The other. The other point I wanted to make is related to Irb.

15:25:54 This is just a I think, an interesting thing to consider that even at the high school level Irb is an important aspects to conducting research, and so is animal care and use considerations with the main math and science Alliance the

15:26:10 Jackson Laboratory is the sponsor. of the main State Science Fair, and for every student, and we we don't have that many gene editing projects that that are proposed.

15:26:21 Because now that requires technology that is usually only available in the research lab, so we don't see too many of those.

15:26:27 But we definitely do see students who are interested in doing projects and involved animals and sometimes human research participants.

15:26:34 And so they follow the exact same process that has been described throughout this presentation.

15:26:41 And so we're really inculcating them as future scientists with the right way to do science from a very early stage.

15:26:50 And I I think I would close there. Thank you for the opportunity.

15:26:56 There were pieces of valuable information. So thank you very much for that.

15:26:58 So what we want to do with the rest of the time was to entertain any questions that folks might have around table about. what was missing, what additional information they'd like brought to future sessions are seller team

15:27:16 from Oprah has been clicking notes as we go along.

15:27:21 But is there anything that hasn't been mentioned of that people would like to have added to the list?

15:27:30 Would it be possible for representative nigger to repeat what's going to be in the next the next year at a slower pace?

15:27:37 Thank thank you. I couldn't write fast enough sure thank you briefly, we have

15:27:53 In a few weeks September seventh There's 2 groups, Group B and C.

15:28:01 Group. B is Gene, editing in the natural world That's things like agriculture, agriculture, the environment, forestry, and fishing group, c.

15:28:13 Which I imagine will be doing the afternoon would be gene editing and the humanities like history.

15:28:20 We'll we'll hear about maliga island we'll hear about eugenics some more ethics faith perspectives

15:28:32 So things like that, Janet so quiet there for a while.

15:28:36 Since that mic doesn't work, unfortunately. these people vacate their seat. I did just want to say that some of that is a little bit dependent on when we can get speakers so it may be that we won't be able to

15:28:49 get the speakers who are recommended, and we may need to shuffle it around.

15:28:53 So if you're watching online or member of the panel don't hold fast to that date as being absolutely the date that that one I was just wanted to get an idea in case it was anything else And that's part of the question can you think

15:29:07 having now gotten our foundational day and underneath us Are there additional things that you'd like to add we should think about it, including

15:29:24 This would be gene editing in systems and institutions.

15:29:28 So looking at State economy business sector. the legal system, education, system and defense in national security, so that you've been talking with the main National Guard and their reach back books. So we have a number of newer programs to keep people in the

15:29:44 State help pay off student loans. that'd be nice to get an update from that DCD.

15:29:53 Is probably actively involved with that work. So, having somebody from the in the department of Economic and community development would be a good thing.

15:30:03 Also in the governor's office about the the future office of the future.

15:30:12 Yeah, yeah, see if they have somebody that they would like to share with us for a portion of the day.

15:30:19 Other thoughts, suggestions, Yes, and 1 one thing we didn't hear about was exactly: how would these treatments be administered?

15:30:28 You know what? what are they? Are they cells that are taken out of the body.

15:30:35 Change reinfused? Are they stem cells that are altered, and then put back in the body?

15:30:40 Are they infusions that go through the body and find their way to where they need to be?

15:30:48 What exactly is the therapy? clinical use of these these new tools?

15:31:03 Yeah, what would it be different? i'm administration so we can better understand that part.

15:31:14 Yes, maybe this is already included in one of those categories.

15:31:19 But you know we've been talking a lot about money and how expensive it is to treat these rare diseases.

15:31:27 I'd like to see if there's a means to make a profit, because sometimes you you could make if you can. If you can cure a middle age, Ladies wrinkles you can make a lot of money and some of that

15:31:39 could offset the cost of treating ware diseases.

15:31:43 You know what I mean, because there's more talking about tax credits and things like that.

15:31:46 I don't know, and and we did talk a little bit about the ethics, you know.

15:31:50 Maybe it's not difficult to treat middle age ladies wrinkles, but I sure wish it were no personally.

15:31:58 But you know that's just an example I just wonder if there is a a profit motive somewhere, because it seems like what we've been talking about so far.

15:32:07 There there really isn't a profit motive because it's not realistic to expect people to pay 22 million dollars for treatment.

15:32:13 They're just not gonna have the customers the profit mode is with Pharma developing this therapy and follow up to the words.

15:32:23 It's all been done by the researchers and they have to make the estimate estimation Well, there's enough payback on the other side and make it worth bringing a market and jumping through all the Fda

15:32:34 hoops, and there usually isn't when it comes to rare diseases.

15:32:38 There's usually

15:32:45 Or I don't know of any way of projecting how many doses of the 2 million dollars they need to see.

15:32:52 Take it back to get back there and invest any individual study of a drug.

15:32:58 Having worked in a drug, a research company, or having worked doing drug research or different comedy.

15:33:05 They invest a whole much millions of dollars upfront and sometimes just walk away from research.

15:33:12 So I think it's not at this level of the research that we're hearing about a university or a Jackson.

15:33:18 Maybe the better people might be at the folks at

15:33:23 I keep wanting to say 0, but it's not so it's I dexter the folks that I dex they have to deal with it, and in animals they might

15:33:34 be able to provide some information about how they do that determination. because they've got to figure out for their patients. They're 4 leg and one.

15:33:43 So right. Maybe There's somebody, an idex who could do a little bit about modeling how they do modeling for bringing drugs to market.

15:33:57 Dr. Chessa

15:34:02 Let me hit unmute there. Yeah, the the thoughts I was having, is. the discussion was happening today. Was you know, sort of as we look at the high cost of these not denying patients care because we can't afford the high cost my

15:34:22 preferred strategy would, B to see if there were mechanisms we could do to lower the cost.

15:34:31 So Generally we take the the cost of these medications as a constant, and we think, figure out what, How can we pay for them?

15:34:41 And you know I prefer saying the cost of these medications is maybe not a constant that there are steps potentially, that the State could take to to to lower the costs that are charged.

15:34:57 And so what what levers are there to pull to try to instead ofize pharmaceutical companies to sort of lower the cost of these medications?

15:35:07 So that's a sort of an area that i'd like to think about.

15:35:10 I do think there are some medications for rare diseases that some pharmaceutical companies have.

15:35:20 You know, quite frankly made help people hostage and made huge profits over rather than sort of getting a mere return on investment.

15:35:29 They've they sort of profit here and so I I sort of wanna make sure that as we're moving forward.

15:35:38 We're thinking about. I sort of how how to manage our relationship with farmarmaceutical companies, and and and and other profit healthcare industry, so that we are able to kind of make these affordable for patients couple of

15:36:01 thoughts. I'm hoping that the new ability to negotiate with maintenance, with medicare part d will help, at least with some of the currently more common drugs I don't know how many years out we need to

15:36:12 be to see it impact rare disease drugs and the research that goes on there.

15:36:19 There are also organizations who've done formulations of cost per additional quality year quality just a life year, and what that's worth, and what that costs per drug and have tried to use that to drive what people are willing to

15:36:38 pay because we certainly can't get it we can't control what the drug companies charge.

15:36:43 We can only control what we pay them and that's only for the people that the State ensures and covers.

15:36:51 So yeah, this has been extended conversation all our time, Laura Laura, Right now.

15:37:01 I just wanted to share something personal related to this and that.

15:37:06 My father in law was diagnosed with Cml.

15:37:10 Which is chronic. Milo the Pm. he is actually was actually lucky enough to have a Philadelphia chromosome, which means that Cleveland which is a targeted therapy for people who have

15:37:22 that particular configuration. what could save his life?

15:37:30 At the time he was diagnosed. Vivac was you know, still on patent Bristol.

15:37:35 Myers Squib is the pharmaceutical company who developed the drug, and the cost was not going to be affordable for him.

15:37:43 So he was faced with the choice of basically succumbing to this disease, or going bankrupt trying to pay for this drug.

15:37:53 Bristol Meyer Squib actually has an affiliated patient assistance program.

15:37:57 I think it's I just looked it up it's the Bristol Myers, with patient assistance foundation he applied to that foundation.

15:38:06 And because of their income that drug was paid for

15:38:09 He remained on Blevik and was subsidized by that program until it went off patent and he could and his medicare. we pay for it, and it has saved his life.

15:38:19 So somehow, in talking with Pharma I mean I don't know how prevalent these kinds of patient assistant programs are.

15:38:27 But I mean it's been amazing at least in this particular situation.

15:38:33 They all have them, and it's because they do well on the margin that they can afford to offer.

15:38:43 The reduced cost is my biased opinion. but all of them have it.

15:38:49 Yes, patty yeah i'll i'll agree with your bias. My bias, too, having taken 50% of my practice was multiple sclerosis.

15:38:59 Very expensive medications, and there was a very narrow group of people who who could get the medications from from the because they they into the

15:39:12 The income guidelines, and it was the people just above it who who suffered, not being able to get the medications, or were in your same position with you know.

15:39:23 Either dealing with the medication with the with the with the cost of it by bet, going bankrupt or or not taking medication at all.

15:39:35 So it just kind of shifts the people who can afford the medication because they were a $100,000 a year.

15:39:45 Of these medications. So so there's that was something else.

15:39:51 I was gonna talk about, but i'll come back to it the cost of medication is one of the most common ones. you hear when you're out campaigning, and in my last practice we had somebody whose full time job it was to work with the

15:40:05 patient assistance programs at the various pharmaceuticals to try and get our patients the next month supply or the month after that, or 3 months supply.

15:40:14 So it's a really common problem, and organizations have committed resources to figure out how to tap that which is kind of unfortunate.

15:40:24 But that's. the way it is yeah any other things we'd like to hear about the haven't been mentioned so far in our future sessions.

15:40:37 Oh, no, oh, very much so if you have any other leads for us additional thoughts. Then I would ask you to get them to our analysts, so they can be shared before the next session.

15:40:53 I'm thinking about the next session and agriculture and Crispr.

15:41:00 And so I thought. For many people this is like a like.

15:41:03 What are we talking about? Exactly like what what you know and so if you i'm sure there'll be some resources sent around.

15:41:10 But one of the big ideas is around climate and altering crops in a way that make them more resistant to changing conditions.

15:41:26 This is about you know the blueberries the developing systems and plants, and in the ocean to capture and hold more carbon carbon sequestration.

15:41:43 So so it's so we're so those are some of the ideas I I imagine we might hear about. but I just I wanted to mention it, because for many of us these are really sort of new concepts and the metal pieces

15:41:57 around Crispr to me are much further along then some of these questions around, you know, altering plants to have more natural pesticides.

15:42:08 Let's say you know there's issues around how you regulate how you might regulate crops that have had Crispr applied to them as opposed to other types of genetic alterations say from other species so i'm just

15:42:25 i'm just putting that out there, and and I I suspect that's there may be other things.

15:42:32 But there's the gear shifts we're gonna make, I think in these different sectors are going to be big. It's not mind boggling enough already.

15:42:46 Any other comments before we wrap up here. and try and respect our 4 o'clock for quick time I see a node from Lauren Hall, with a suggestion for the do CEO at Jack, says maybe be a resource

15:43:03 to understand the workings of Pharma, so we can pursue that a bit.

15:43:09 So thank you specifically to Dana. Where are you going for?

15:43:16 Educating us this this morning. I learned a lot, and I appreciate the the way that you did that.

15:43:26 Thank you again to the staff for pulling us all together to make it a very productive day.

15:43:31 I i'm grateful and thank you all those people on the horseshoe who are willing to commit significant time and energy in the next 2 and a half months next 2 months to this work cause as we've

15:43:47 heard It's kind of important so you have my personal gratitude.

15:43:54 I'm, looking forward to working with you some more any other comments from you, Denver.

15:44:01 Okay, I always before I adjourn a meeting, I always turn to the analyst and say, What have I left out?

15:44:08 What do you need for me? Do you have a sense of what the questions are

15:44:17 You're all set to that being the case let's adjourn until our next scheduled meeting.

15:44:24 Yeah, Thank you. Hi: Everyone: Thank you so long. Thank you.