2015 SENATE HUMAN SERVICES

SB 2259

2015 SENATE STANDING COMMITTEE MINUTES

Human Services Committee
Red River Room, State Capitol

SB 2259
2/2/2015
22945

☐ Subcommittee
☐ Conference Committee

Committee Clerk Signature Donald Mueller

Explanation or reason for introduction of bill/resolution:

A bill relating to the use of experimental drugs

Minutes:

Attach #1: Testimony by Sen. Tim Mathern
Attach #2: Testimony by Laura Kulsrud
Attach #3: Testimony by Donene Feist

Senator Tim Mathern introduced SB 2259 to the Senate Human Services Committee. This establishes the parameters for those who are terminally ill can receive treatment that has not been approved by Federal Food and Drug Administration (FDA). (3:43) (attach #1)

Senator Dever stated that it appears the barriers are federal, not state. Are you suggesting we can circumvent those?

Senator Mathern understands that other states around the nation have been able to craft legislation that creates a narrow door where in this type of care can be provided even though we have federal law. This is the challenge. Some of the other states call this "the right to try" legislation. The attempt is recognizing the federal control, but within that control the state can proceed.

Senator Heckaman testified IN FAVOR of SB 2259. She will bring concurrent resolution in the future to discuss this issue also. (ends 6:15)

Laura Kulsrud, mother of three children with PKAN, spoke IN FAVOR of SB 2259. (attach #2) (6:35-17:00) Ms. Kulsrud also asked to amend the bill to add language this includes any drug that is currently under investigation in an FDA clinical trial but has not been approved for use by FDA. This language would keep this bill on par with those already passed in other states.

Senator Dever stated that it never ceases to amaze him where God matches special children with special parents. Other states have passed similar legislation. Senator Dever stated that he got the impression that this in-itself doesn't do it. Would this allow that or would it send a message to the FDA?

Ms. Kulsrud responded that there were come changes that were made in the language, but we need to put the wording back in the language so we align with the other states. If this drug does get out there into a trial use, this language might work.

Robin Anderson, friend of Ms. Kulsrud testified. The process with FDA, we submitted a physician IND, which was denied. Part of the reason for denial was stating they didn't have a commercial IND from the drug company already in place, they didn't have the information to approve the physicians IND. So now the drug company is telling the family and their investors that they plan to have their commercial IND in place within the first six months of 2015. When we initially looked at the bill and the draft, that language was in there, stating being part of phase 1 of an FDA trial. We had asked that that be removed because RE02 drug is not in phase 1 of an FDA trial. So at this point, it would not help the family. But knowing that it will be in phase 1 of an FDA trial in the very near future, then our intent is that we would resubmit the physician's IND and knowing that it will be in a trial already, if they didn't approve the physician's IND the third time around, the Right to Try bill, SB 2259, would help in those efforts. In raising awareness, if it doesn't help treat the boys, it may change when there are no other options when there could be a drug out there to help them.

Senator Howard Anderson, Jr. stated with the suggested amendment, the drug has to be in an approved investigational status, which this one is not at this time. You also indicated other countries. Is it in investigational stage in other countries?

Ms. Anderson restated that it will be in a trial in the first 6 months of this year. It may be May 2015 before that's the case. The patient being treated in Europe, the European union approved a trial for an individual under compassionate care and is being treated in another country.

Chairman Judy Lee asked is it possible to be treated in another country.

Ms. Kulsrud indicated they could be treated, but shelf life is only 3 weeks, and would have to travel every 3 weeks. It's not feasible.

Senator Howard Anderson, Jr. asked if the drug company going into a clinical trial knew that these individuals were available and met the qualifications of the study, and knew this is forthcoming, they should have interest. Senator Howard Anderson, Jr. understands the concern.

Senator Axness indicated that the language was previously in the bill, and then removed.

Ms. Anderson indicated they did have it, but removed because it wouldn't help immediately this family. However, they want it back in to be consistent with other states and then would have the chance to get it approved. The drug company commercial IND trial will be for adults only, and that is where we would still need the physician's IND trial so it can used to treat children. The compassionate use case in Europe was an adult, it was not a child. The drug company did offer to take the children to Europe for treatment. They are considering doing this, until there was a change in the drug company, change in management.

Senator Axness asked the intern, Femi, to provide the original amendment (01 version), confirmed by Chairman Judy Lee.

Donene Feist, Director for Family Voices of North Dakota, spoke IN FAVOR of SB 2259. (attach #3) (27:00-29:30)

Senator Howard Anderson, Jr. stated what we are asking in the bill is for the FDA to approve a compassionate care provision. Not available until drug is under investigation clinical trial. We are looking to the future and not trying to solve the current or past problem. Is that correct?

Ms. Feist indicated that this will hopefully remove the barrier so when the drug is available, they can use it.

Senator Warner is it typical to segregate adult research versus children research?

Senator Howard Anderson, Jr. indicated yes. A drug company doesn't want the liability of what will happen to children, so they will do it on healthy adults first.

Senator Warner asked is that initial stage to test the toxicity of the drug rather than it's curative properties? Separate process for therapeutic levels?

Senator Howard Anderson, Jr. answered the first stage of use in humans is to use the drug in healthy adults. Then if it is proved to be safe, we can go to people with who might have the disease and see if it is effective or not.

Senator Warner so we are asking here is to allow its therapeutic use to be tested in a compassionate care setting at a time when it is being tested for toxicity in adults?

Senator Howard Anderson, Jr. answered yes.

Chairman Judy Lee stated her sympathy to the family. She further stated the children's rights include being protected by the people who serve in the legislature. The FDA may be the bad player in this case, the intent is to make sure that people who are given drugs are going to have them done safely and hopefully in a curative fashion.

OPPOSITION TO SB 2259
No opposing testimony

NEUTRAL TO SB 2259

Chairman Judy Lee invited pharmacists to discuss about drug review.

Mr. Mark Hardy, Executive Director of North Dakota Board of Pharmacy to the podium.

Senator Howard Anderson, Jr. asked Mr. Hardy to describe the phases of a drug trial.

Mr. Hardy explained the different phases. The phase 1 trial is the pre-clinical trial for toxicity, and is for healthy adults. Phase 2 trials continues and expands to measure the effectiveness of the drug. Senator Howard Anderson, Jr. phase 3 is fine tune it, identify the proper dosage, and start marketing the drug.

Robin Anderson provided more information. In testing for PKAN, very seldom do they live to be an adult. This will be handled a little differently because of this. Onset to death is usually 10 years. There are about 1,000 PKAN patients in United States. They estimate 5,000-10,000 in the world. When this disease is injected into laboratory rats, the rats die within 3 days. With treatment of drug, the rats return to normal state. With this drug, it is hard to find adults with this disease.

Senator Dever asked if this drug would cure the disease or delay the symptoms.

Ms. Anderson indicated hopeful it will cure the disease. In this family situation, they are hoping that it stops the progression of the disease. They are seeing reverse in Cyprus case.

Chairman Judy Lee asked about Australia

Ms. Anderson indicated they never did get to the point of approval. There are three brothers also in that country, so they continue to look to start a trial.

Chairman Judy Lee other locations?

Ms. Anderson said they looked at Canada, but they are still working on it there as well. It takes less time in Canada to go through the approval process.

Brendan Joyce, Pharmacist for Medicaid, Department of Human Services, spoke, but had no prepared testimony. Mr. Joyce wanted to make the committee aware of the wording in the bill to ensure there are not consequences. In Section 23.08.04, the section regarding the word "access", access is tied very tightly with payment by CMS and others. While this bill indicates that insurance does not have to pay, the CMS world the word "access", where because we don't pay for experimental, we are blocking access. Medicaid does not pay for experimental. We do not pay for out of country experiences either.

Closed Public Hearing.

2015 SENATE STANDING COMMITTEE MINUTES

Human Services Committee
Red River Room, State Capitol

SB 2259
2/2/2015
23012

☐ Subcommittee
☐ Conference Committee

Committee Clerk Signature Donald Mueller

Explanation or reason for introduction of bill/resolution:

A bill relating to the use of experimental drugs

Minutes:

Attach #1: Proposed Amendment in draft format

Received the original version of amendment (15.0251.01000). (attach #1). The committee reviewed the draft bill as proposed amended.

(1:27)
V. Chairman Oley Larsen thought they were talking about the avenue of a resolution before the bill. Even if we pass the bill, the federal guidelines trump it and they would need the resolution to pass before the bill could take effect.

Chairman Judy Lee the resolution won't do anything unless except urge the federal government to do it. There is a concurrent resolution that is related to SCR 4016. It will not be heard today, because not scheduled, but it is in relation to SB 2259.

Senator Howard Anderson, Jr. indicated that if we passed this legislation, it would have no effect.

Chairman Judy Lee stated there is one human being who has used this drug in the world, a person in Cyprus. Chairman Judy Lee understands how challenging this. She provided examples of her husband was sick, and this is nothing something easily dealt with the family, but we also have to recognize our responsibility for all children, we should not put children in that high risk position. It's one thing for an adult and another for a child. We all want this to work, but worried about what this could do. **Senator Dever** indicated if it was his child, he would be doing the same thing, as did the whole committee, but they all understand the risk of harm to children.

Senator Howard Anderson, Jr. top of page 2 of the draft bill, it has to be through phase 1 of clinical trial. This is the phase where they determine the drug is safe - it won't kill the healthy adults we give it to. His gut feeling is support resolution but not the bill.

Chairman Judy Lee indicated that she would like to visit with the Attorney General's office for input.

Senator Howard Anderson, Jr. stated that at some point we need to address the question about the term "access" so maybe we could change that word so we don't get Medicaid in trouble. Line 15, Page 4. **Chairman Judy Lee** indicated that we would have to amend the other language that is on the original Section 2, sub-section 2, with version 1, subsection 2 and choose a different word than "access." Asked Maggie Anderson (DHS) if there is a better word or phrase, and let Femi know what the change could be.

2015 SENATE STANDING COMMITTEE MINUTES

Human Services Committee
Red River Room, State Capitol

SB 2259
2/9/2015
23446

☐ Subcommittee
☐ Conference Committee

Committee Clerk Signature Donald J. Mueller

Explanation or reason for introduction of bill/resolution:

A bill relating to the use of experimental drugs

Minutes:

Attach #1: Email from Megan Houn
Attach #2: SB 2259 Draft Bill with proposed amendment
Attach #3: email from Maggie Anderson with proposed amendment

NOTE: This bill is related to SCR 4016. **Chairman Judy Lee** recapped the SB 2259 hearing and information. Also distributed email from Megan Houn (attach #1)

Senator Howard Anderson, Jr. asked if there a proposed amendment from Department of Human Services regarding medical services and reimbursement? **Chairman Judy Lee** indicated yes, and that they were previously distributed. Chairman Judy Lee read through the previously discussed amendments.

Distributed SB 2259 Draft Bill with proposed amendment (Attach #2)
Distributed email from Maggie Anderson (DHS) with proposed amendment (attach #3)

Senator Howard Anderson, Jr. moved to ADOPT AMENDMENT to SB 2259, as provided by Maggie Anderson (DHS) - "Nothing in this section requires payment for experimental drugs under state's medical assistance program." The motion was seconded by **Senator Axness**.

Discussion
(9:30)

Senator Dever reminded the committee that Brendan Joyce was concerned about the word "access".

Committee indicated that draft bill with amendments and the Department of Human Services amendment should address that.

Chairman Judy Lee might want to add a sentence "or insurance provider"... if we are eliminating Medicaid as an entity that has to pay, what about the other insurance company? Should they also be not included from responsibility?

Marny Walth from Sanford Health. Yes, we should follow the same path as Medicaid. We should not have to pay.

Chairman Judy Lee should we use the word "third party" rather than medical assistance program? With Department of Human Services, the agreement on the terminology will be "all payer sources".

Senator Dever top of page 4, subsection 4, if an eligible patient dies while being treated by an investigational drug product (Senator Dever read directly from the bill), patient heirs are not liable for outstanding debt related to the treatment or lack of insurance due to the treatment. So who is liable?

Chairman Judy Lee stated it would appear the company and provider?

Senator Warner bottom of page 3, line 23, if pre-existing condition insurance covers that, but if new condition arises or side effect of treatment, they are not obligated to pay for that. That is an older version. Senator Warner then read from the bill. (14:32)

Chairman Judy Lee stated she didn't think that's what the intention was.

Megan Houn, Blue Cross Blue Shield , the way it is written, it could turn into a mandate in how it is written. It is difficult who is going to pay for it if something catastrophic goes wrong. Not intended to make it a mandate, but it kind of does. There is still federal preemption, so if FDA doesn't approve, Feds are still going to pre-empt.

Chairman Judy Lee stated perhaps it is not worth fixing the bill up.

Senator Howard Anderson, Jr. moved previous question on the amendment.

Roll Call Vote to Amend SB 2259
6 Yes, 0 No, 0 Absent

Senator Howard Anderson, Jr. stated he was never in favor of the concept for approval of drugs, but resolution is fine. I don't expect that won't accomplish much either. To say we are setting up the scenario up in North Dakota, we do have empathy to the family, but we likely will not accomplish anything.

Senator Howard Anderson, Jr. made a motion to DO NOT PASS as Amended. The motion was seconded by **V. Chairman Oley Larsen**.

Discussion
Senator Dever indicated the bill could actually put up additional barriers. He agrees with the motion, as much as everyone wishes they could do something for the family.

Roll Call Vote to DO NOT PASS as Amended
5 Yes, 1 No, 0 Absent

Senator Howard Anderson, Jr. will carry SB 2259 to the floor.

(19:20) Note: the remaining part of recording 23446 is the vote on the SCR 4016, which is related to SB 2259.

15.0251.02001
Title.03000

Adopted by the Human Services Committee

February 9, 2015

2/9/15

PROPOSED AMENDMENTS TO SENATE BILL NO. 2259

Page 1, line 1, replace "23-28" with "23-48"

Page 2, line 6, after "investigation" insert "in a United States food and drug administration-approved clinical trial"

Page 4, after line 17 insert "This section does not require payment for experimental drugs under this state's medical assistance program or from other payer sources."

Renumber accordingly

Date: 02/09 2015
Roll Call Vote #: 1

2015 SENATE STANDING COMMITTEE
ROLL CALL VOTES
BILL/RESOLUTION NO. SB 2259

Senate Human Services Committee

☐ Subcommittee

Amendment LC# or Description: 15.0251.02001 Title 03000 Will not allow Reimbursement by all payer sources

Recommendation: ☑ Adopt Amendment
☐ Do Pass ☐ Do Not Pass ☐ Without Committee Recommendation
☐ As Amended ☐ Rerefer to Appropriations
☐ Place on Consent Calendar

Other Actions: ☐ Reconsider ☐ _____

Motion Made By Anderson Seconded By Axness

Senators	Yes	No	Senators	Yes	No
Senator Judy Lee (Chairman)	✓		Senator Tyler Axness	✓	
Senator Oley Larson (V-Chair)	✓		Senator John M. Warner	✓	
Senator Howard C. Anderson, Jr.	✓				
Senator Dick Dever	✓				

Total (Yes) 6 No 0

Absent 0

Floor Assignment _____

If the vote is on an amendment, briefly indicate intent:

Date: 02/09 2015
Roll Call Vote #: 2

2015 SENATE STANDING COMMITTEE
ROLL CALL VOTES
BILL/RESOLUTION NO. SB2259

Senate Human Services Committee

☐ Subcommittee

Amendment LC# or Description: 15.0251.02001 Title. 03000

Recommendation: ☐ Adopt Amendment
☐ Do Pass ☒ Do Not Pass ☐ Without Committee Recommendation
☒ As Amended ☐ Rerefer to Appropriations
☐ Place on Consent Calendar

Other Actions: ☐ Reconsider ☐ _____

Motion Made By Anderson Seconded By Larsen

Senators	Yes	No	Senators	Yes	No
Senator Judy Lee (Chairman)	✓		Senator Tyler Axness	✓	
Senator Oley Larson (V-Chair)	✓		Senator John M. Warner		✓
Senator Howard C. Anderson, Jr.	✓				
Senator Dick Dever	✓				

Total (Yes) 5 No 1

Absent 0

Floor Assignment Anderson

If the vote is on an amendment, briefly indicate intent:

REPORT OF STANDING COMMITTEE

SB 2259: Human Services Committee (Sen. J. Lee, Chairman) recommends **AMENDMENTS AS FOLLOWS** and when so amended, recommends **DO NOT PASS** (5 YEAS, 1 NAYS, 0 ABSENT AND NOT VOTING). SB 2259 was placed on the Sixth order on the calendar.

Page 1, line 1, replace "23-28" with "23-48"

Page 2, line 6, after "investigation" insert "in a United States food and drug administration-approved clinical trial"

Page 4, after line 17 insert "This section does not require payment for experimental drugs under this state's medical assistance program or from other payer sources."

Renumber accordingly

2015 HOUSE HUMAN SERVICES

SB 2259

2015 HOUSE STANDING COMMITTEE MINUTES

Human Services Committee
Fort Union Room, State Capitol

SB 2259
3/10/2015
Job #24577

☐ Subcommittee
☐ Conference Committee

Committee Clerk Signature Kenneth M. Tordel

Explanation or reason for introduction of bill/resolution:

Relating to the use of experimental drugs

Minutes: Testimonies #1, #2, #2B, #3, #4.

Chairman Weisz opened the hearing on SB 2259.

Sen. Tim Mathern: Testified in support of the bill. (See Testimony #1)

Rep. Chet Pollert: I give my full support to SB 2259. The Kulsruds are actually from the northern part of Foster County and the southern part of Eddy County, and that's why you'll see Sen. Heckaman here as well. I ask the chairman if he would oblige that I testify real quick and get back to committee because I'm actually working on the DHS budgets and detailing right now. My full support for SB 2259.

Sen. Joan Heckaman: Went through the bill. (See Testimony #2)

11:20

Chairman Weisz: Do you want to respond to a question on definition?

Sen. Heckamann: Yes. I will try, but I think we have the national representative here, who has been in on the development of the bills as they've been around into other states, but I can give it a shot.

Chairman Weisz: My question is, you have your 1-6 all requirements that have to be met under the definition of eligible. Correct? But then, in the case of why you brought the bill, wouldn't they automatically be eliminated under if there has to be a clinical trial within 100 miles of the patient's home address?

Sen. Heckamann: I think that means if there is a trial, the patient should be participating in that, but I'll let the representative answer that. When I visited with him this morning, I said I don't want to make any statement that is not accurate on this bill. That's probably a question I better step back from.

Sen. Terry Wanzek: I want to suppress my support for this bill. The family that has been referenced, the Kulsruds, live within our district. I'm just coming at it from the angle of putting myself in their shoes. Being a parent, I just can't imagine the emotions that they must be going through in dealing with this situation. This is an effort to try and help them. I don't understand all the details, and that's why we have committees with folks like you, and hopefully we can work through that and figure it out. I understand that the FDA might take action, by the FDA to ultimately really help them, but I see this as a message from our state to the FDA. It's a message from us that we support this family. I also think of my brother, who farms with me, last year lost his wife to pancreatic cancer. I know, in visiting with him, we were talking about this issue and this family, and I know he shared with me, when you come down to the end, and all your options are exhausted, you just about will consider anything to try and save that loved one's life. So I'm here in support of the family as much as I am in support of the bill. And I hope you give it a thorough hearing and strong consideration.

Rep. Kylie Oversen: District 42. I was proud to be asked to be a sponsor of this bill, and I think if it can provide easier access to these medications for families that are really struggling, that would be a great step for our state to take. And I would ask for your support, and would be happy to answer any questions.

Laura Kulsrud, Parent of PKAN Children: Testified in support of the bill. (See Testimony #2-B)

24:50

Chairman Weisz: You indicated in your testimony that the company is more than willing to give you the drug. I assume that if we pass this legislation, all it really will do is send a message to the FDA because they still wouldn't be able to give you the drug?

Kulsrud: Normally you can allow a physician IND (Investigational New Drug) before a commercial IND is filed. There has been proof of it. We had proof of other drugs they had done that. With this, the FDA was saying, no; this review committee. They kept on us that we need a commercial IND submitted before the physician, even though we went back in their guidelines and found that you don't have to. So now the drug company is working to get their commercial IND submitted. They wanted more testing, is what the FDA was saying, so they did the testing now, and they're working to compile the data and submit it in. Once it's submitted, it goes to Phase 1, which we all know, and it goes into that safety trial. But we possibly could get this if it goes into that first phase, then we could get it without having to be in a so-called trial, and have to go through placebo, and wait. There is talk they might just start it with adults only. Therefore, our children wouldn't be able to. This disease often onsets when they are children; they don't make it to adulthood. So there isn't very many adults that have this disease.

Chairman Weisz: From your perspective, if we pass this legislation for you, will it speed up the process for you? Do you believe it will?

Kulsrud: Possibly. I don't know. I can't look into the future.

Chairman Weisz: But that's your hope, that it will put additional pressure on the FDA?

Kulsrud: Yes.
27:30

Craig Handzlik, State Policy Coordinator, Goldwater Institute: testified in support of the bill. (See Testimony #3)
42:20

Rep. D. Anderson: You've kind of described Phase 1. Usually, how many phases are there before a product gets on the market? And is there an average timeline before that happens?

Handzlik: The FDA clinical trials process is a four-part process. There are three phases. The first phase, which tests for safety and toxicity, typically will take somewhere between one to three years to complete. The second and third phases, they hone in on dosages, possible side effects, and they will take an additional three to four years apiece. Once a medication or a device has passed the third phase of the FDA clinical trials, it goes on to what's called the approvals phase, the process is the FDA is analyzing all the data and deciding whether it'll be approved. The total time, It really depends on who you ask, but it takes anywhere between 8-15 years for a medication to from first phase to approval. And the cost there, depending on who you ask, as well, is somewhere in the neighborhood of $1-billion to $2-billion. What Right To Try does, is, it aims to get those medications to terminally ill patients, once we've realized that they're not toxic, and they're safe to ingest.

Rep. Mooney: My question goes back to some testimony provided by Sen. Heckaman earlier today. It was bullet point #14 that said, the insurer may deny coverage from the time the patient begins using the product through a period not to exceed 6 months from the time the product is no longer used by the patient. So I'm wondering if you could expand just a little bit about that. What all that means.

Handzlik: In every state, the language is a little different. The intent, though, is the same everywhere. The intent of the language with regard to liability protections for insurance companies essentially is, the bottom line is, insurance companies are not required to cover the costs related to an investigational medication. The language here, with the six months, essentially the intent here is that we all realize when you take an investigational medication, there is some risk. And we'd like the risk to be solely based on the informed consent of the patient. And, if an insurance company says that while you're taking this investigational medication, we're not going to cover the cost for your underlying illness's regular coverage, or your insurance policy, we would be fine with that. The patient has made the decision that they're going to give this medication a try, and that this would be one of the bullet points in their informed consent.

Rep. Mooney: So, really what you're saying then, is that it provides an out for the insurance company, with direct relation to the investigation piece. But if they still have other conditions, outside of the investigation, those would still be insured. Correct?

Handzlik: Yes. For instance, if a patient took an investigational medication, had positive reaction to it, but yet it still didn't cure their diabetes, in six months, according to the way North Dakota's law is written, the insurance company would hopefully provide coverage for the insulin treatment, or what have you, unrelated to the investigational medication.

Rep. Mooney: But we're not leaving people susceptible that their diabetes is no longer covered?

Handzlik: That's the intent of the bill; that we're not leaving insureds susceptible.

Rep. Rich Becker: Because of your long-term involvement and your knowledge of the FDA, do you ever perceive the FDA changing their views? They have their reasons for being as restrictive, some would say obstinate, as they are, but when you hear the stories that we've heard today, you just have to question. My question to you is quite simply, do you see in years to come that the FDA will ever change any of their policies?

Handzlik: If you follow the issue like I do, we are starting to see the FDA budging, thinking, realizing they've left out a really important, but small population of the medical community. The most recent example of how the FDA has shown some movement is in their Compassionate Use Expanded Access Program. Admittedly, they have listed on the initial application form that this paperwork takes on average 100 physician hours to complete. And that's the most public portion of the process. What that means to you and I is that if I've got a terminal illness, and you're my physician, that you have to set aside 2 ½ entire work weeks just to fill out the application for me, one patient. Now you know that physicians don't have time to spend more than about four minutes with you in the office. Setting aside 2 ½ weeks is a pretty tall order. And that's the shortest part of the expanded access program. So the FDA most recently seeing that 29 states have their own version of Right To Try, moving through the legislature, that five of those states, the bill is sitting on the governor's desk, ten additional states in those 29 have passed at least one chamber, Right To Try is happening. It has passed in five states already, and patients, doctors, manufacturers are working together to get through the process, get the medications to the patients that they need. And the FDA has realized that look, our process is long, our process is demanding. I'm not sure it was designed for someone who has two months to live, because the first part of our process is 100 hours of physician paperwork, the manufacturer has an additional burden of paperwork at the same time. That goes to the FDA, the FDA then reviews all of that paperwork and has 30 days to issue some sort of a ruling. However, if they've got questions on either branch of the 100 hours of paperwork, the doctor or the manufacturing company, they can send that paperwork back and the 30-day clock starts again. I don't know about you, but when I was renting a car last night and I filled out a page and a half of paperwork of all information I knew off the top of my head, there were questions about it. You can only imagine what kind of questions would arise from 100 hours of scientific, physician paperwork. So, they've said that they're going to shorten their form. They're going to take it from 100 hours to 45 minutes. Which is a humongous in the eyes of those of us who are helping to push this legislation. However, a simple form change is not necessarily the solution we're looking for here. We're looking for the ability for patients and doctors to work together to try to save patients' lives instead of to have to beg the Federal government to do so.

52:15

Donene Feist: Director for Family Voices of ND testified in support of the bill. (See Testimony #4)

56:51

Shane Wendel: Pharmacist, testified in support of the bill. I am the Kolsrud's family pharmacist. Their grandmother is my pharmacy technician. So I know their circumstance very well. Being their pharmacist and being a pharmacist in general in Carrington, anytime somebody takes a drug, there is a risk to taking it. Just about every medication that you take can kill you. And then we have risk benefit. So we take drugs with the hope that it's going to do more good than harm. And that's the FDA's job; to help us protect ourselves from these drugs. And in this circumstance, with the Kulsruds, the risk is quite severe. If they do not take the drug, they're going to die from their disease. To me, the benefit of this drug, of them getting it, and this legislature passing the bill, to somehow send a message to increase their cause of getting this drug, could be life-saving. So, looking at it from a pharmacist's perspective, of risk benefit, to me the drug doesn't offer any additional risk at all. The risk is already built in, and they have a terminal disease. The benefit could turn around and save their life. As the medical community, that's what we try to do is improve people's health and life, and risk benefit is always considered before they take a medication.

58:34

Jack McDonald: On behalf of America's Health Insurance Plans testified in support of the bill. AHIP generally supports these bills with the provision that there is not a mandated payments provision, and there is a line on your bill, on page 4, lines 19 and 20, and so, we do support the bill. We urge that you give this a Do Pass, and if you have any questions, I'd be glad to try to answer them.

Chairman Weisz: Others in support of SB 2259? Are there those here in opposition to SB 2259?

Robert Harms, On behalf of CVS Health: Our only concern is the provision with regard to mandated health coverage, and if the committee would work on some language, particularly with regard to page 3, lines 22 through the balance of that page. To clarify the testimony that was given earlier by the Heartland Institute that the bill isn't designed to mandate insurance payments, I think we'd be OK with it. But that is a section we've had two or three lawyers go through it, and we all come up with different answers to it. We think the way it's drafted, does present some problems, and we'd recommend the committee work on that. That's our only over-riding concern to the bill.

Rod St. Aubyn For the Pharmacy Care Management Assn.: Our concerns are similar to what Mr. Harms had said. I want to go back to a question that Rep. Mooney asked, and I think it's a very legitimate question. If you go on page 3, lines 26-30, and Mr. Harms has already talked a lot about this: it is very confusing. An insurer may deny coverage to an eligible patient from the time the eligible patient begins the use of investigational drug

biologic product, etc. through a period not to exceed six months from the time the investigational drug, whatever, no longer is used by the eligible patient. So let's just use this in real time frames. Let's say that I'm a patient and I take this on Jan. 1 of this year, and I take it until June 30. So, as this is clearly written, it sure may deny coverage, what type of coverage? If I have diabetes during that period of time, now during that period of time, the insurer does not have to cover any of those? As I see this, there would be no coverage whatsoever for the period of Jan 1 through Dec. 31st in my example. Now, that may not be the intent of this, but this language is very troubling that I think really needs to be reviewed. The other thing is, If the intent is just for this particular drug, you don't have to cover. Let me play this scenario by you: I try this investigational drug for six months; all of a sudden my condition goes into remission, which would be wonderful, so now six months, I'm not taking it any more, then after that period, the conditions come back. Now does the insurer have to cover that investigational drug? The way this is worded, it says the insurer may deny, however, during that period; only that period. Does that imply then, after that period, that they may have to? So, it's real questionable, just the way this is worded. I know what they talk about, the intent, and if somehow the wording can be changed to reflect the intent, I think that would be better for the committee. I would be willing to answer any questions.

Rep. Oversen: So, to clarify, if we work on that language, would the providers be comfortable not covering the investigational drug, but would be comfortable covering other prescriptions that the patient is already on for diabetes or other pre-existing conditions? So it needs to be written that their coverage is not mandated just for that specific drug that they're trying?

St. Aubyn: I'm not sure than an insurer, and this would probably be a better question to ask the insurance department, but I'm not sure that they can exclude other benefits that are in their health plan. From my standpoint, I think that would be a question to ask the health insurance department.

(Not Audible, Not Registered) Director of Government Relations- BC/BS: We are not opposed to the bill. I would provide a little clarifying comment, and to what was said prior. And, also, as a mother of three children, I can completely understand where you're coming from. My family also has Familiar ALS, so this is a very important bill to folks in North Dakota. When I just spoke to our general counsel shortly, they have the same concerns around the same section that both Rod St. Aubyn and Mr. Harms had mentioned. Section 3 on page 3, A through maybe Section 4; Our general counsel felt that if that was deleted, the supportive statement that they had gave on page 4 at the bottom, talks about it doesn't require payment for experimental drugs, that might actually clarify the intent a little bit better. Just get rid of the health insurance language, and leave the intent with it doesn't require payment by payers. I think that reflects the intent of the bill more clearly.

Chairman Weisz: Any opposition to SB 2259? Hearing none.

Chairman Weisz closed the hearing on SB 2259.

2015 HOUSE STANDING COMMITTEE MINUTES

Human Services Committee
Fort Union Room, State Capitol

SB 2259
3/11/2015
Job #24700

☐ Subcommittee
☐ Conference Committee

Committee Clerk Signature Vicky Crabtree

Minutes:

Chairman Weisz: SB 2259. The insurance providers had some issues with the language. I did visit with a couple and my suggestion would be is that we remove all of 3, a, b and c on page 3. If we remove that there is language in subsection 4, e on page 2 that says they are not obligated to pay for the investigational drug or product. With that in there the normal insurance law applies so you don't have worry about all the questions that came up relative to 3. I believe that fixes the issues.

Rep. Fehr: I move your amendment.

Rep. Porter: Second

VOICE VOTE: MOTION CARRIED

Rep. Hofstad: I move a Do Pass on SB2259 as amended.

Rep. Seibel: Second.

Rep. Fehr: I heard that FDA rulings may preclude some things and I don't understand that.

Chairman Weisz: It is my understanding that this bill isn't going to do anything accept maybe help push the FDA into addressing the issue.

Rep. Porter: Wouldn't it be prudent to put an amendment on the bottom of this bill that sent a letter notifying the FDA that we did it.

Chairman Weisz: That might have more effect than anything.

Rep. Porter: Add language down at the bottom of the resolution that copies of this are sent to our congressional delegation and a copy to the FDA. I make them do a return receipt so somebody has to act on it.

Rep. Hofstad: I'll retract me motion.

Rep. Seibel: Second.

Rep. Porter: I would further amend by adding a section 2 that would send notification to our congressional delegation and to the FDA that the provisions of this law have been enacted by the State of ND by certified mail return receipt requested.

Rep. Fehr: Second.

VOICE VOTE: MOTION CARRIED

Rep. Hofstad: I move a Do Pass as amended on engrossed SB 2259.

Rep. Seibel: Second.

ROLL CALL VOTE: 13 y 0 n 0 absent

MOTION CARRIED

Bill Carrier: Rep. Mooney

15.0251.03001
Title.04000

Adopted by the Human Services Committee 3/11/15

March 11, 2015

PROPOSED AMENDMENTS TO ENGROSSED SENATE BILL NO. 2259

Page 1, line 2, after "drugs" insert "; and to provide for a notification by secretary of state"

Page 3, remove lines 22 through 30

Page 4, remove lines 1 and 2

Page 4, line 3, replace "4." with "3."

Page 4, after line 28, insert:

"**SECTION 2. NOTIFICATION BY SECRETARY OF STATE.** The secretary of state shall notify the federal food and drug administration and the North Dakota congressional delegation of this bill by sending a copy of this bill upon filing with the secretary of state."

Renumber accordingly

Date: 3-11-15
Roll Call Vote #: 1

2015 HOUSE STANDING COMMITTEE
ROLL CALL VOTES
BILL/RESOLUTION NO. 2259

House Human Services Committee

☐ Subcommittee

Amendment LC# or Description: See description below

Recommendation: ☒ Adopt Amendment
☐ Do Pass ☐ Do Not Pass ☐ Without Committee Recommendation
☐ As Amended ☐ Rerefer to Appropriations
☐ Place on Consent Calendar

Other Actions: ☐ Reconsider ☐ _____

Motion Made By Rep. Fehr Seconded By Rep. Porter

Representatives	Yes	No	Representatives	Yes	No
Chairman Weisz			Rep. Mooney		
Vice-Chair Hofstad			Rep. Muscha		
Rep. Bert Anderson			Rep. Oversen		
Rep. Dick Anderson					
Rep. Rich S. Becker					
Rep. Damschen					
Rep. Fehr					
Rep. Kiefert					
Rep. Porter					
Rep. Seibel					

Voice Vote
Motion Carried

Total (Yes) _____ No _____

Absent _____

Floor Assignment _____

If the vote is on an amendment, briefly indicate intent:

Remove lines 22 through 30 on page 3
Remove lines 1 and 2 on page 4

Date: 3-11-15
Roll Call Vote #: 2

2015 HOUSE STANDING COMMITTEE
ROLL CALL VOTES
BILL/RESOLUTION NO. 2259

House Human Services Committee

☐ Subcommittee

Amendment LC# or Description: See description below

Recommendation: ☒ Adopt Amendment
☐ Do Pass ☐ Do Not Pass ☐ Without Committee Recommendation
☐ As Amended ☐ Rerefer to Appropriations
☐ Place on Consent Calendar

Other Actions: ☐ Reconsider ☐ _____

Motion Made By Rep. Porter Seconded By Rep. Fehr

Representatives	Yes	No	Representatives	Yes	No
Chairman Weisz			Rep. Mooney		
Vice-Chair Hofstad			Rep. Muscha		
Rep. Bert Anderson			Rep. Oversen		
Rep. Dick Anderson					
Rep. Rich S. Becker					
Rep. Damschen					
Rep. Fehr					
Rep. Kiefert					
Rep. Porter					
Rep. Seibel					

Voice Vote
Motion Carried

Total (Yes) _____ No _____

Absent _____

Floor Assignment _____

If the vote is on an amendment, briefly indicate intent:

add language and a Section 2 that the state of ND will notify FDA of what we did and get a return receipt from them.

Date: 3-11-15
Roll Call Vote #: 3

2015 HOUSE STANDING COMMITTEE
ROLL CALL VOTES
BILL/RESOLUTION NO. 2259

House Human Services Committee

☐ Subcommittee

Amendment LC# or Description: _____

Recommendation: ☐ Adopt Amendment
☒ Do Pass ☐ Do Not Pass ☐ Without Committee Recommendation
☒ As Amended ☐ Rerefer to Appropriations
☐ Place on Consent Calendar

Other Actions: ☐ Reconsider ☐ _____

Motion Made By Rep. Hofstad Seconded By Rep. Seibel

Representatives	Yes	No	Representatives	Yes	No
Chairman Weisz	✓		Rep. Mooney	✓	
Vice-Chair Hofstad	✓		Rep. Muscha	✓	
Rep. Bert Anderson	✓		Rep. Oversen	✓	
Rep. Dick Anderson	✓				
Rep. Rich S. Becker	✓				
Rep. Damschen	✓				
Rep. Fehr	✓				
Rep. Kiefert	✓				
Rep. Porter	✓				
Rep. Seibel	✓				

Total (Yes) 13 No 0

Absent 0

Floor Assignment Rep. Mooney

If the vote is on an amendment, briefly indicate intent:

REPORT OF STANDING COMMITTEE

SB 2259, as engrossed: Human Services Committee (Rep. Weisz, Chairman) recommends **AMENDMENTS AS FOLLOWS** and when so amended, recommends **DO PASS** (13 YEAS, 0 NAYS, 0 ABSENT AND NOT VOTING). Engrossed SB 2259 was placed on the Sixth order on the calendar.

Page 1, line 2, after "drugs" insert "; and to provide for a notification by secretary of state"

Page 3, remove lines 22 through 30

Page 4, remove lines 1 and 2

Page 4, line 3, replace "4." with "3."

Page 4, after line 28, insert:

"SECTION 2. NOTIFICATION BY SECRETARY OF STATE. The secretary of state shall notify the federal food and drug administration and the North Dakota congressional delegation of this bill by sending a copy of this bill upon filing with the secretary of state."

Renumber accordingly

2015 CONFERENCE COMMITTEE

SB 2259

2015 SENATE STANDING COMMITTEE MINUTES

Human Services Committee
Red River Room, State Capitol

SB 2259
4/7/2015
25864

☐ Subcommittee
☒ Conference Committee

Committee Clerk Signature Donald Mueller

Explanation or reason for introduction of bill/resolution:

A bill relating to the use of experimental drugs

Minutes: No attachments

The following conference committee members were present for SB 2259 on April 7, 2015, 10:30 a.m.
Senator Howard Anderson, Jr., Senator Lee, Senator Warner
Representative Kiefert, Representative Seibel, Representative Oversen

Senator Howard Anderson, Jr. asked **Representative Kiefert** to explain the amendments.

Representative Kiefert indicated that he hasn't had a chance to look over the bill to see what the issues were this morning. One of the main issues is on Page 3, lines 22 through 30. We didn't want to have the insurance companies to pay for the experimental treatment. Our concern in committee was that the language was unclear to having them pay for the regular treatment that they would need that was not related to the experimental treatment. He thought they had left that part in.

Representative Oversen stated the section that we removed, end of page 3 and top of page 4, we thought that overall insurance companies shouldn't have to pay for the experimental drug but otherwise won't be able to drop coverage for other existing conditions, and they can't do that already. We removed the sections, and on page 4, lines 19 and 20, it reads, "This section does not require payment for experimental drugs under the state's medical assistance program or from other payer sources." This will cover insurance companies that they wouldn't be mandated to provide coverage for experimental drugs, and we thought it was okay to remove the other language so there wasn't confusion.

Senator Howard Anderson, Jr. indicated that the amendments were suggested by Blue Cross Blue Shield, and they felt comfortable with the last section that you just referred to would mean they wouldn't have to pay for it. There was a little confusion with line 26, 27, 28 on page 3. The concern was that they might be able to drop the coverage for a broken

leg, even though you were taking some experimental drug. We didn't think that should be in there. He did talk to the sponsors on the Senate side and all three of them indicated they were comfortable with the amendments. He did hear from the Goldwater Institute that was behind the bill, and they also said this final version would be fine.

Chairman Judy Lee apologized for being late, and the record shows she is present.

Senator Warner asked a question, returning to the bottom of page 3, line 26. Senator Howard Anderson, Jr. had mentioned the broken leg or some other related thing. Do you understand this that they could deny coverage for treating side effects of the experimental drug? Not just paying for the drug, but say it caused nausea, or some other problems such as hemorrhaging.

Representative Oversen indicated they didn't discuss the side effects portion of that. We discussed pre-existing conditions that were already covered.

Representative Seibel indicated that when he reads page 2, line 27 through 29, subsection e, that covers that - "the health insurer and provider are not obligated to pay for any care or treatments consequent to the use of the investigational drug, biological product, or device."

Senator Warner added consequent should be caused by rather than subsequent to. **Representative Seibel** confirmed.

Representative Kiefert said he has some concern about somebody that has a diabetic problem that is not related to cancer treatment, that all of a sudden they have to address that issue to. He was under the understanding that this was in the bill. That it protected the person from a broken leg. It could exclude them from services.

Senator Howard Anderson, Jr. indicated he thinks that was one of the reasons you removed the language on lines 26 through 30. It gave the impression that they could exclude services. Now with that out, he doesn't think that is a problem.

Representative Kiefert indicated that he was reading it the wrong way.

Senator Lee requested a two minute recess in the hallway for the Senators.

(9:10)
Senator Warner moved that the Senate accede to the House Amendments for SB 2259. The motion was seconded by **Senator Lee**. No discussion.

Roll Call Vote
Senators: 3 Yes, 0 No, 0 Absent
Representatives: 3 Yes, 0 No, 0 Absent

Senator Howard Anderson, Jr. will carry to the Senate floor.
Representative Mooney will carry to the House floor.

Date: 4/7/2015
Roll Call Vote #: 1

2015 SENATE CONFERENCE COMMITTEE
ROLL CALL VOTES

BILL/RESOLUTION NO. SB 2259 as engrossed

Senate "Enter committee name" Committee

Action Taken
- ☒ **SENATE accede to House Amendments**
- ☐ **SENATE accede to House Amendments and further amend**
- ☐ **HOUSE recede from House amendments**
- ☐ **HOUSE recede from House amendments and amend as follows**

☐ **Unable to agree**, recommends that the committee be discharged and a new committee be appointed

Motion Made by: Sen. Warner Seconded by: Sen. Lee

Senators	07			Yes	No		Representatives	07			Yes	No
Senator Anderson	x			x			Representative Kiefert	x			x	
Senator Lee	x			x			Representative Seibel	x			x	
Senator Warner	x			x			Representative Kylie	x			x	
Total Senate Vote				3	0		Total Rep. Vote				3	0

Vote Count Yes: 6 No: 0 Absent: 0

Senate Carrier Sen. Anderson House Carrier Rep. Mooney

LC Number _____ . _____ of amendment

LC Number _____ . _____ of engrossment

Emergency clause added or deleted

Statement of purpose of amendment

REPORT OF CONFERENCE COMMITTEE

SB 2259, as engrossed: Your conference committee (Sens. Anderson, J. Lee, Warner and Reps. Kiefert, Seibel, Oversen) recommends that the **SENATE ACCEDE** to the House amendments as printed on SJ page 831 and place SB 2259 on the Seventh order.

Engrossed SB 2259 was placed on the Seventh order of business on the calendar.

2015 TESTIMONY

SB 2259

SB2259
Attach #1
02/02/15
JH 22945

SB 2259
February 2, 2015

Madam Chairman Lee and members of the Senate Human Service Committee,

My name is Tim Mathern. I am the senator from District 11 in Fargo and here to introduce SB 2259.

In short, this bill establishes the parameters within which a person who is terminally ill may receive treatment with care that has not yet be approved for general use by the United States Food and Drug Administration.

I introduced this bill at the request of a family who is here to testify. I do not know the family personally but I decided to proceed when I learned that they were in a critical situation and that they were being supported in their efforts by Family Voices, an organization that advocates for families in a constructive manner. Facing the death of a family member and knowing something might help but that something is just out of reach is a painful place to be. I hope we can help this family and others out there to take a well supervised and contained risk when no other options are available.

I drafted the bill in as narrow a manner to be attentive to the risks involved but wide enough that the present situation might benefit.

Madam Chairman and members of the Committee, I know you are often presented with difficult stories and asked to make public policy to protect all of the citizens and yet meet a specific need. As such as you hear all the testimony I trust your judgments as to any amendments you might suggest.

I ask for a do pass recommendation from your committee.

02/2/2015
SB2259 Attach#2
#22945

Testimony on SB 2259
2015 Legislative Session
Feb 2, 2015
Sen. Lee, Senate Human Services Chairperson

Senator Lee and Members of the Senate Human Services Committee,

My name is Laura Kulsrud and I am the mom of three young boys that have been diagnosed with the deadly disease PKAN (Pantothenate Kinase-Associated Neurodegeneration). My husband, Jay, and our boys, Lane-13, Tanner-11, and Ty-9 live in Grace City, ND. During the fall of 2011, Lane started to experience problems with slurred speech and balance leading him to stumble and fall often. After several tests, Doctors diagnosed him with the PKAN. It is a genetic disease where a gene is mutated and which causes iron to accumulate in the basil ganglia of the brain. The iron build-up causes many neurological symptoms to occur. Since this is a genetic disease, we tested our two younger boys. The test results came back positive that both Tanner and Ty have PKAN, but at the time were symptom free. As you can imagine, the news was devastating as there is no cure and our sons are going to die at an early age if a cure is not found. Shortly after the diagnosis, Tanner began experiencing some of the same symptoms as Lane, which have progressively become worse. To this date, Ty remains symptom free, but he often wonders when this ugly disease will strike him. Ty is now at the same age the other two boys were at onset. Can you imagine the thoughts that go through his head as he watches his brothers struggle each day? We remained hopeful during the past few years that there would be a cure to get rid of this disease and that our boys would once again be able to experience life the same way their peers do.

Over a year ago, we learned of a company called Retrophin that had been researching a new drug called RE-024, with much success in treating PKAN when tested in animals. RE-024 is a derivative of vitamin B5. People with PKAN cannot process vitamin B5 into phospho-B5, which is essential to live. Without phospho-B5, iron builds up at the base of the brain which results in the same neuorological problems our boys experience. When tested on rats and monkeys, RE-024 has shown to return them to a normal state. They have tested toxicity and have found none. RE-024 has been proven to be very safe in the lab.

Last spring, our doctor submitted a Physician's Investigational New Drug (or IND) trial application for treatment of RE-024 in our boy's names under the FDA Compassionate Use law. We were hopeful that the boys would be allowed to start this trial shortly after submitting the application. On April 10, 2014, we received word that the FDA had denied our application. They suggested we re-submit the application which we did. Shortly after that submission we received word that once again we were denied approval from FDA. This was a blow to our family. We researched many other options including treatment in Australia and Europe.

In May 2014, Retrophin began a trial in Europe treating one patient with RE-024. The patient was unable to walk unassisted at the time and now walks freely. Which tells us that this drug is not only safe in animals but also in humans. Granted, this is one case, but when you have three boys with this deadly disease, one is enough for us. So with excitement comes more frustration, knowing that there is an opportunity to help our boys get better, but that we have an FDA that continues to deny us the only option available to us now.

Since April 2014, we have been lobbying to get our physician IND approved. We have met with four individuals at FDA headquarters in Maryland, met in Washington, DC with six United States Senators and 1 Congressman, of which all seven have been lobbying on our behalf in DC for approval. Our North Dakota Senators and Congressman have had two phone calls directly with FDA Commissioner, Margaret Hamburg, urging her to approve this IND. Commissioner Hamburg has the power to overturn the FDA review committee's decision with one phone call, yet we continue to be denied.

Other efforts have included the creation of an online petition urging the FDA to approve the IND along with time spent being interviewed on many local radio talk shows and local news stations. All of this has brought awareness to the issue but we still have not been able to proceed with the Physician's IND to treat the boys with RE-024. Time is of the essence, as each day they lose a bit more of their normal self. We are not asking the FDA to approve RE-024 for general use, only to allow it to be taken in a clinical trial by our children who are counting on this drug. It's shocking to me that we have a drug company willing to give us the drug, a doctor, who is seen as an expert on the disease, willing to administer it, parents willing to sign any documentation allowing treatment of our kids and boys who are begging for the drug so that they have a chance to live a normal life. Yet, individuals working from an office in Washington, DC, continue to deny treatment and tell us that they know better for our three terminally ill kids than their doctor, the drug company, their parents and the boys who are living with the effects of this disease daily.

I am urging you to support Senate Bill 2259. Several states have passed similar bills and it is raising awareness at the FDA that there is a need to revamp the current approval process for Investigational New Drug Trials. We are a country of great progress in the medical field, yet we sometimes fall behind other countries that are forward thinking in allowing new treatments in which the patient has no other option. Many families like ours are trying so desperately to get treatments to save their loved ones but the current bureaucratic processes at the FDA are leaving us with little hope. The need for change in expanded access needs to happen soon. Medical advances are on the rise and we will see more and more cases where a medication is available that could potentially help a terminally ill patient but they can't get through the red tape to save their own life. The time for change is now. We do not want anyone else to go through the same struggles that we have endured. We will continue to fight, not only for PKAN patients, but all patients that should have the right to try a drug when they have no other options.

As for our boys, Lane is having many physical troubles. Recently, he is loosing the ability to tie his shoes because his hands are starting to fail him. Writing in school is a challenge because of his hands. His speech is failing him also because of dystonia in his mouth, he is loosing the ability to communicate like he should be able to. He continues to fall down numerous times a day, on some occasions hitting his head. He needs RE-024!

Tanner is struggling with the use of his arms and hands. This was once a kid who was very active and in a short amount of time he lost the ability to do many everyday tasks. He can no longer dress himself, put shoes on, nor tie them. Because of his arms being spastic he is off balance and falls often. He also has dystonia in his mouth- speaking and eating is difficult. He also needs RE-024! As for all the boys, they take massive amounts of medicine daily trying to slow the progression of this disease and to help relax the spastic muscles in their bodies. Tanner takes the most pills, 27 pills daily! If we could have a chance at this new drug, we could eliminate all of them and take just one.

As a parent, one of the hardest things to watch is your kids struggling to do everyday tasks that they once could do easily. What may be worse, is listening to your children beg and plead daily for a medicine that we believe could reverse their symptoms and possibly take them back to a physical state that would allow them a normal life, once again. My boys have the same dreams as any other boy their age. They want to play football and basketball. They want to ride horse and show their animals at the state fair. They want to grow up and start families of their own. So many of those dreams have already been taken away from them, but they have an opportunity to achieve many of those dreams with your help. You can give them that chance. Without RE-024 those dreams will never happen. I beg you to support this legislation and give my family the hope that we are desperately seeking.

I thank you for your time today.

Laura Kulsrud
Grace City, ND
701.653.5930

#22945 02/02/15
Attach #3
SB2259

Testimony on SB 2259
2015 Legislative Session
Feb 2, 2015
Sen. Lee, Senate Human Services Chairperson

Senator Lee and Members of the Senate Human Services Committee,

My name is Donene Feist, I am the Director for Family Voices of North Dakota. I come before you today to request support for SB 2259.

Family Voices of North Dakota is statewide health information and education center who serves families of children with special health care needs in ND. We provide emotional and informational support to many families across North Dakota who have a child who has an ongoing medical diagnosis such as asthma, heart conditions, terminal illness, physical disabilities, and emotional/behavioral issues. Our staff, who all are parents to children with special health care needs, provides assistance to families by helping them access and navigate services.

FVND has assisted families who face decisions each day that are very difficult decisions to make. When you have a child with a terminal illness you want to make the best decisions for your family and child. This is not done in isolation. Many families work diligently to receive the treatments needed for their child. Families cannot wait, expanded access to these necessary drugs are vital for families.

States have been leading the cause for change regarding Investigational Drug Trials in encouraging the FDA to change their policies regarding the approval process. My hope is that this committee will see the need to do the same.

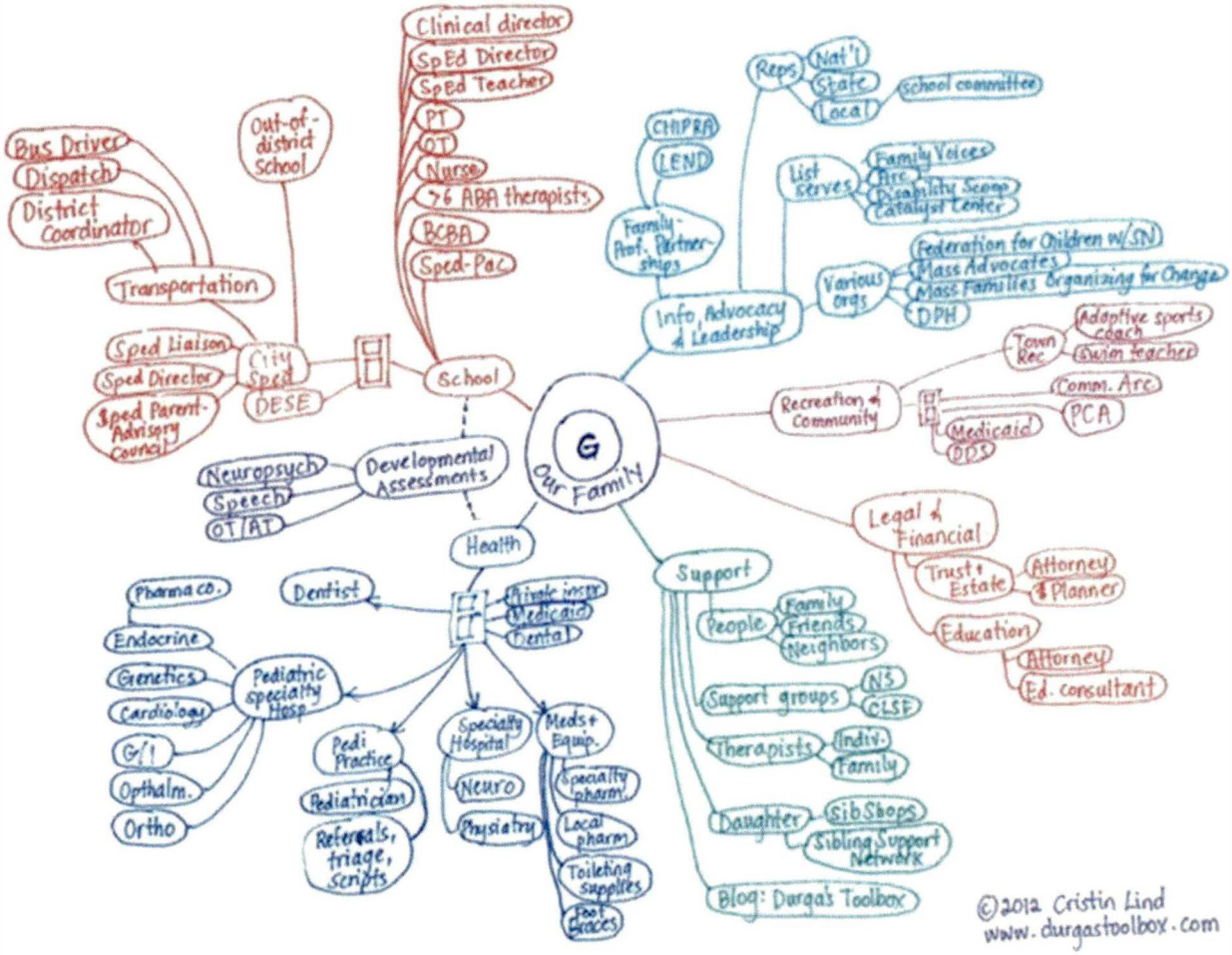
In assisting other families across North Dakota, we know that the process and barriers to approving lifesaving medication is most difficult. Families should not have to wait and work through all of the barriers in order to assist their serious or terminally ill child. Often, there isn't the time.

We also know that through medical advances, there could be a medication available tomorrow. But with the barriers, families are still not able to participate. We can do better.

Not only will this bill help the children who face a terminal illness, it will also assist the adults. Individuals with cancer, ALS, MS to name a few.

It strikes me as we just had the ALS challenge that it seemed many participated in. This is clearly something you can do as lawmakers to make a difference for the individuals and families who face a serious or terminal illness.

In the diagram below, I am leaving you with this visual of all of the services families encounter when they have a child with special health care needs. Families are left to navigate systems either on their own or with the help of an organization like FVND. As you can see from the diagram, many services are in silos. Having a child with a serious and terminal illness would be another layer of complexity that the family would need to endure.



As policymakers, what would you do if your grandchild or child had a serious/terminal illness that you knew there was a drug that may be able to assist?

What if it was your spouse? I think we can all agree on what we would do. Changes can happen, you can make it happen.

You can send a message today to the FDA that changes need to happen.

In closing…."**Let us remember as each of us makes decisions that will affect children—whether we are parents, educators, health professionals, or government officials—it is our duty to consider if that decision either affirms or denies a child's most basic human rights.**"

Thank you for your consideration

Donene Feist
Family Voices of ND
PO Box 163
Edgeley, ND 58433
701-493-2634
fvnd@drtel.net

15.0251.01000

Sixty-fourth
Legislative Assembly
of North Dakota

BILL NO.

SB2259
Attach #1
12/02/15 Committee
JH 23012

Introduced by

Senator Mathern

A BILL for an Act to create and enact chapter 23-28 of the North Dakota Century Code, relating to the use of experimental drugs.

BE IT ENACTED BY THE LEGISLATIVE ASSEMBLY OF NORTH DAKOTA:

SECTION 1. Chapter 23-48 of the North Dakota Century Code is created and enacted as follows:

23-48-01. Definitions.

As used in this chapter, unless the context otherwise requires:

1. a. "Eligible patient" means an individual who:
   (1) Has a terminal illness that is attested to by the patient's treating physician;
   (2) Considered all other treatment options currently approved by the United States food and drug administration;
   (3) If there is a clinical trial for the terminal illness within one hundred miles of the patient's home address for the terminal illness, is unable to participate in the clinical trial or within one week of completion of the clinical trial application process is not accepted to the clinical trial;
   (4) Has a recommendation from the patient's treating physician for an investigational drug, biological product, or device;
   (5) Has given written, informed consent for the use of the investigational drug, biological product, or device or, if the patient is a minor or lacks the mental capacity to provide informed consent, a parent or legal guardian has given written, informed consent on the patient's behalf; and
   (6) Has documentation by the patient's treating physician the patient meets the requirements of this subdivision.

b. The term does not include an individual treated as an inpatient in a hospital licensed under chapter 23-16.

2. "Investigational drug, biological product, or device" means a drug, biological product, or device that has successfully completed phase one of a clinical trial but has not yet been approved for general use by the United States food and drug administration and remains under investigation in a United States food and drug administration-approved clinical trial.

3. "Terminal illness" means a disease that, without life-sustaining procedures, will soon result in death or a state of permanent unconsciousness from which recovery is unlikely.

4. "Written, informed consent" means a written document signed by the patient or the patient's parent or legal guardian and attested to by the patient's treating physician and by a witness which:

a. Explains the currently approved products and treatments for the terminal illness from which the patient suffers;

b. Attests to the fact the patient concurs with the patient's treating physician in believing that all currently approved and conventionally recognized treatments are unlikely to prolong the patient's life;

c. Identifies the specific proposed investigational drug, biological product, or device the patient is seeking to use;

d. Describes the potentially best and worst outcomes of using the investigational drug, biological product, or device with a realistic description of the most likely outcome, including the possibility that new, unanticipated, different, or worse symptoms might result, and that death could be hastened by the proposed treatment, based on the treating physician's knowledge of the proposed treatment in conjunction with an awareness of the patient's condition;

e. States the patient's health insurer and provider are not obligated to pay for any care or treatments consequent to the use of the investigational drug, biological product, or device;

f. States the patient's eligibility for hospice care may be withdrawn if the patient begins curative treatment and that hospice care may be reinstated if the curative treatment ends and the patient meets hospice eligibility requirements;

g. States in-home health care may be denied if treatment begins; and

h. Attests that the patient understands the patient is liable for all expenses consequent to the use of the investigational drug, biological product, or device, and that this liability may extend to the patient's estate, unless a contract between the patient and the manufacturer of the drug, biological product, or device states otherwise.

23-48-02. Drug manufacturers - Availability of investigational drugs, biological products, or devices - Costs - Insurance coverage.

1. A manufacturer of an investigational drug, biological product, or device may make available the manufacturer's investigational drug, biological product, or device to an eligible patient pursuant to this chapter. This chapter does not require that a manufacturer make available to an eligible patient an investigational drug, biological product, or device.

2. A manufacturer may:

   a. Provide to an eligible patient an investigational drug, biological product, or device without receiving compensation; or

   b. Require an eligible patient to pay the costs of, or the costs associated with, the manufacture of the investigational drug, biological product, or device.

3. a. This chapter does not expand a health insurance mandate provided for under chapter 26.1-36.

   b. An insurer may provide coverage for the cost of an investigational drug, biological product, or device.

   c. An insurer may deny coverage to an eligible patient from the time the eligible patient begins use of the investigational drug, biologic product, or device through a period not to exceed six months from the time the investigational drug, biologic product, or device is no longer used by the eligible patient. However, under this subdivision, coverage may not be denied for a preexisting condition or for

coverage for benefits that commenced before the time the eligible patient began use of the drug, biologic product or device.

4. If an eligible patient dies while being treated by an investigational drug, biological product, or device, the eligible patient's heirs are not liable for any outstanding debt related to the treatment or lack of insurance due to the treatment.

23-48-03. Action against health care provider's license or medicare certification prohibited.

Notwithstanding any other law, a licensing board may not revoke, fail to renew, suspend, or take any action against a health care provider's license issued in this state, based solely on the health care provider's recommendations to an eligible patient regarding access to or treatment with an investigational drug, biological product, or device, if the recommendations are consistent with medical standards of care. Action against a health care provider's medicare certification based solely on the health care provider's recommendation that a patient have access to an investigational drug, biological product, or device is prohibited.

23-48-04. Access to investigational drugs, biological products, and devices.

An official, employee, or agent of this state may not block or attempt to block an eligible patient's access to an investigational drug, biological product, or device. Counseling, advice, or a recommendation consistent with medical standards of care from a licensed health care provider is not a violation of this section.

23-48-05. Cause of action not created.

This chapter does not create a private cause of action against a manufacturer of an investigational drug, biological product, or device or against any other person involved in the care of an eligible patient using the investigational drug, biological product, or device, for any harm done to the eligible patient resulting from the investigational drug, biological product, or device, if the manufacturer or other person complied in good faith with the terms of this chapter. However, this chapter does not limit a private cause of action against a manufacturer or other person if there was a failure to exercise reasonable care.

02/09/15
JH 23446

SB2259
Megan Houn
emailed 02/05/15
Attach #1

From: Megan Houn [mailto:Megan.Houn@bcbsnd.com]
Sent: Thursday, February 05, 2015 12:50 PM
To: Lee, Judy E.
Subject: 2259

Hi Senator Lee,
It was lovely to visit with you on Tuesday evening. Below I've pasted some of our concerns with Senate Bill 2259 from our friend Bob Stroup. I tried to pick the high points from the multiple paragraphs sent my way.

"If I understand this language correctly, the initial sentence only allows an insurance company to deny coverage for a drug, biologic or device as being experimental from the time of its use extending for 6 months following the initial denial, but then it needs to be covered (or can no longer be denied) if I read this correctly. Additionally, the second sentence in this provision imparts a preexisting condition restriction on health insurers like BCBSND and does not permit BCBSND to deny coverage for an experimental drug, biologic or device "for benefits that commenced before the time the eligible patient began use of the drug, biologic product or device." A reasonable reading of this language appears to prohibit an insurance company from denying reimbursement for services and benefits for experimental drugs, biologics and devices if the covered individual began receiving these before coverage for benefits began.

Similarly, if Legal's interpretation of Section 23-48-02(3)(c), N.D.C.C., is correct, or even reasonable without clarification or explanation as to the intent of this language and its meaning, this would appear to create mandated coverage for experimental drugs, biologics and devices under certain circumstances despite what the language of the bill suggests. These are high-dollar benefits and services and would increase the costs of health insurance in North Dakota significantly.

Even without the curious language included in SB 2259 and its meaning or intent, the current bill does not make exceptions for any complimentary health care services and benefits incidental to the coverage for these experimental drug benefits, such as costs for in-patient treatment, professional charges, and other benefits and services extended to a covered member as a result of the administration of experimental drugs, biologics and devices. The same consideration holds true in regard to complications caused by the use of experimental drugs, biologics and devices. Such ancillary and incidental costs are a real factor that would cause increased costs in administering health insurance in North Dakota."

Honestly, that's the short version. ☺ If you would like the longer version, or have further questions, please don't hesitate.
Kind regards,
Megan

Megan Houn
Director, Government Relations
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Attach #2
SB 2259
02/09/15
JH 23446

SB 2259 DRAFT AMENDMENT

A BILL for an Act to create and enact chapter ~~23-28~~ 23-48 of the North Dakota Century Code, relating to the use of experimental drugs.

BE IT ENACTED BY THE LEGISLATIVE ASSEMBLY OF NORTH DAKOTA:

SECTION 1. Chapter 23-48 of the North Dakota Century Code is created and enacted as follows:

23-48-01. Definitions.

As used in this chapter, unless the context otherwise requires:

1. a. "Eligible patient" means an individual who:

(1) Has a terminal illness that is attested to by the patient's treating physician;

(2) Considered all other treatment options currently approved by the United States food and drug administration;

(3) If there is a clinical trial for the terminal illness within one hundred miles of the patient's home address for the terminal illness, is unable to participate in the clinical trial or within one week of completion of the clinical trial application process is not accepted to the clinical trial;

(4) Has a recommendation from the patient's treating physician for an investigational drug, biological product, or device;

(5) Has given written, informed consent for the use of the investigational drug, biological product, or device or, if the patient is a minor or lacks the mental capacity to provide informed consent, a parent or legal guardian has given written, informed consent on the patient's behalf; and

(6) Has documentation by the patient's treating physician the patient meets the requirements of this subdivision.

b. The term does not include an individual treated as an inpatient in a hospital licensed under chapter 23-16.

2. "Investigational drug, biological product, or device" means a drug, biological product, or device that has successfully completed phase one of a clinical trial but has not yet been approved for general use by the United States food and drug administration and remains under investigation in a United States food and drug administration-approved clinical trial.

3. "Terminal illness" means a disease that, without life-sustaining procedures, will soon result in death or a state of permanent unconsciousness from which recovery is unlikely.

4. "Written, informed consent" means a written document signed by the patient or the patient's parent or legal guardian and attested to by the patient's treating physician and by a witness which:

a. Explains the currently approved products and treatments for the terminal illness from which the patient suffers;

b. Attests to the fact the patient concurs with the patient's treating physician in believing that all currently approved and conventionally recognized treatments are unlikely to prolong the patient's life;

c. Identifies the specific proposed investigational drug, biological product, or device the patient is seeking to use;

d. Describes the potentially best and worst outcomes of using the investigational drug, biological product, or device with a realistic description of the most likely

outcome, including the possibility that new, unanticipated, different, or worse symptoms might result, and that death could be hastened by the proposed treatment, based on the treating physician's knowledge of the proposed treatment in conjunction with an awareness of the patient's condition;

e. States the patient's health insurer and provider are not obligated to pay for any care or treatments consequent to the use of the investigational drug, biological product, or device;

f. States the patient's eligibility for hospice care may be withdrawn if the patient begins curative treatment and that hospice care may be reinstated if the curative treatment ends and the patient meets hospice eligibility requirements;

g. States in-home health care may be denied if treatment begins; and

h. Attests that the patient understands the patient is liable for all expenses consequent to the use of the investigational drug, biological product, or device, and that this liability may extend to the patient's estate, unless a contract between the patient and the manufacturer of the drug, biological product, or device states otherwise.

23-48-02. Drug manufacturers-Availability of investigational drugs, biological products, or devices-Costs-Insurance coverage.

1. A manufacturer of an investigational drug, biological product, or device may make available the manufacturer's investigational drug, biological product, or device to an eligible patient pursuant to this chapter. This chapter does not require that a manufacturer make available to an eligible patient an investigational drug, biological product, or device.

2. A manufacturer may:

a. Provide to an eligible patient an investigational drug, biological product, or device without receiving compensation; or

b. Require an eligible patient to pay the costs of, or the costs associated with, the manufacture of the investigational drug, biological product, or device.

3. a. This chapter does not expand a health insurance mandate provided for under chapter 26.1-36.

b. An insurer may provide coverage for the cost of an investigational drug, biological product, or device.

c. An insurer may deny coverage to an eligible patient from the time the eligible patient begins use of the investigational drug, biologic product, or device through a period not to exceed six months from the time the investigational drug, biologic product, or device is no longer used by the eligible patient. However, under this subdivision, coverage may not be denied for a preexisting condition or for coverage for benefits that commenced before the time the eligible patient began use of the drug, biologic product or device.

4. If an eligible patient dies while being treated by an investigational drug, biological product, or device, the eligible patient's heirs are not liable for any outstanding debt related to the treatment or lack of insurance due to the treatment.

23-48-03. Action against health care provider's license or medicare certification prohibited. Notwithstanding any other law, a licensing board may not revoke, fail to renew, suspend, or take any action against a health care provider's license issued in this state, based solely on the health care provider's recommendations to an eligible patient regarding access to or treatment with an

investigational drug, biological product, or device, if the recommendations are consistent with medical standards of care. Action against a health care provider's medicare certification based solely on the health care provider's recommendation that a patient have access to an investigational drug, biological product, or device is prohibited.

23-48-04. Access to investigational drugs, biological products, and devices.

An official, employee, or agent of this state may not block or attempt to block an eligible patient's access to an investigational drug, biological product, or device. Counseling, advice, or a recommendation consistent with medical standards of care from a licensed health care provider is not a violation of this section. Nothing in this section requires payment for experimental drugs under the state's medical assistance program.

23-48-05. Cause of action not created.

This chapter does not create a private cause of action against a manufacturer of an investigational drug, biological product, or device or against any other person involved in the care of an eligible patient using the investigational drug, biological product, or device, for any harm done to the eligible patient resulting from the investigational drug, biological product, or device, if the manufacturer or other person complied in good faith with the terms of this chapter. However, this chapter does not limit a private cause of action against a manufacturer or other person if there was a failure to exercise reasonable care.

SB 2259
Attach #3
02/09/15
J# 23446

NDLA, S HMS - Mueller, Don

From: Anderson, Maggie D.
Sent: Monday, February 02, 2015 7:32 PM
To: NDLA, Intern 01 - Adisa, Femi
Cc: NDLA, S HMS - Mueller, Don; Joyce, Brendan
Subject: SB 2259 (Inv Drugs)

Femi,
Per the request of Senator Anderson to address the concern raised by the Department of Human Services in the hearing today on SB 2259, we offer the following amendment:

Line 17 after "section." Add: "Nothing in this section requires payment for experimental drugs under the state's medical assistance program."

Please let us know if you have questions.
Thank you.
Maggie

Maggie Anderson
ND Department of Human Services
manderson@nd.gov
701-328-2538

SB 2259
3/10/2015 #1

SB 2259
March 10, 2015

Chairman Weisz and members of the House Human Service Committee,

My name is Tim Mathern. I am the senator from District 11 in Fargo and here to introduce SB 2259.

In short, this bill establishes the parameters within which a person who is terminally ill may receive treatment with care that has not yet be approved for general use by the United States Food and Drug Administration.

I introduced this bill at the request of a family who is here to testify. I did not know the family personally but I decided to proceed when I learned that they were in a critical situation and that they were being supported in their efforts by Family Voices, an organization that advocates for families. Facing the death of a family member and knowing something might help but "that something" is just out of reach is a painful place to be. I trust we can help this family and others to take a well supervised and contained risk when no other options are available.

I drafted the bill in as narrow a manner to be attentive to the risks involved but wide enough that the present family situation before us might benefit.

Madam Chairman and members of the Committee, I know you are often presented with difficult stories and asked to make public policy to protect all of the citizens and yet meet a specific need.

I ask for a do pass recommendation from your committee.

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#2

SB 2259
March 10, 2015

Chairman Weisz and Members of the House Human Services Committee: I am Senator Joan Heckaman from New Rockford and I represent District 23. I am here to support SB 2259.

SB 2259 is important to all of us in North Dakota because of the opportunity it provides for us to address the rights we have as citizens. And with those rights come responsibilities.

This morning you will hear Laura Kulsrud tell the story she and her family have endured the last few years as Jay and Laura's 3 boys have a progressive degenerative nerve disorder-PKAN. She will tell her story that not only relates the physical toll this disease has had on the boys, but also the emotional toll of trying to find a solution.

I believe the solution may be here in SB 2259. 5 states already have this law on the books. 26 states have introduced the "Right to Try" bill this year.

I would like to review the bill with this abbreviated summary.

1. Eligible patients must have a terminal illness and have exhausted all other treatment options.
2. Have a clinical trial within 100 miles of the patient's home or is not accepted into a trial.
3. Has to have a recommendation from the patient's treating physician to use the product.
4. Parental consent in writing is mandated if the patient is a minor.
5. The product must have successfully completed phase one of a clinical trial but not yet been approved by the FDA.
6. Patient's health insurer and provider are not obligated to pay for any care or treatments consequent to the use of an investigational product.
7. Hospice care may be withdrawn if the patient begins curative treatment under this bill.
8. In home health care may be denied if treatment begins.

9. Manufacturer may make the produce available to the patient with this chapter but it does not mandate the manufacturer has to do that.
10. The manufacturer may make the product available without receiving compensation.
11. The patient may have to pay the costs of or the cost associated with the manufacture of the product.
12. Does not mandate an expansion of a health insurance mandate.
13. But the insurer may provide coverage for the product.
14. The insurer may deny coverage from the time the patient begins using the produce through a period not to exceed 6 months from the time the product is no longer used by the patient.
15. If the patient dies while being treated, the patient's heirs are not liable for any outstanding debt related to treatment or lack of insurance due to treatment.
16. Licensing board may not revoke a provider's license based solely on the provider's recommendation for the patient to receive the product.
17. The access may not be blocked by an official, employee, or agent of the state.
18. Relieves action against the manufacturer for any harm to the patient from the product.

I won't take any more time because I want you to hear from the Kulsrud family. This bill is not only for them. It is also for many other families who find a loved one with no other options. I believe SB 2259 will provide the important options that many are waiting for.

I ask for your support for SB 2259.

Senator Joan Heckaman

SB 2259
3/10/2015
#2-B

Testimony on SB 2259
2015 Legislative Session
March 10, 2015
Rep. Weisz, House Human Services Chairperson

Representative Robin Weisz and Members of the House Human Services Committee,

My name is Laura Kulsrud and I am the mom of three young boys that have been diagnosed with the deadly disease PKAN (Pantothenate Kinase-Associated Neurodegeneration). My husband, Jay, and our boys, Lane-13, Tanner-11, and Ty-9 live in Grace City, ND. During the fall of 2011, Lane started to experience problems with slurred speech and balance leading him to stumble and fall often. After several tests, Doctors diagnosed him with the PKAN. It is a genetic disease where a gene is mutated and which causes iron to accumulate in the basil ganglia of the brain. The iron build-up causes many neurological symptoms to occur. Since this is a genetic disease, we tested our two younger boys. The test results came back positive that both Tanner and Ty have PKAN, but at the time were symptom free. As you can imagine, the news was devastating as there is no cure and our sons are going to die at an early age if a cure is not found. Shortly after the diagnosis, Tanner began experiencing some of the same symptoms as Lane, which have progressively become worse. To this date, Ty remains symptom free, but he often wonders when this ugly disease will strike him. Ty is now at the same age the other two boys were at onset. Can you imagine the thoughts that go through his head as he watches his brothers struggle each day? We remained hopeful during the past few years that there would be a cure to get rid of this disease and that our boys would once again be able to experience life the same way their peers do.

Over a year ago, we learned of a company called Retrophin that had been researching a new drug called RE-024, with much success in treating PKAN when tested in animals. RE-024 is a derivative of vitamin B5. People with PKAN cannot process vitamin B5 into phospho-B5, which is essential to live. Without phospho-B5, iron builds up at the base of the brain which results in the same neuorological problems our boys experience. When tested on rats and monkeys, RE-024 has shown to return them to a normal state. They have tested toxicity and have found none. RE-024 has been proven to be very safe in the lab.

Last spring, our doctor submitted a Physician's Investigational New Drug (or IND) trial application for treatment of RE-024 in our boy's names under the FDA Compassionate Use law. We were hopeful that the boys would be allowed to start this trial shortly after submitting the application. On April 10, 2014, we received word that the FDA had denied our application. They suggested we re-submit the application which we did. Shortly after that submission we received word that once again we were denied approval from FDA. This was a blow to our family. We researched many other options including treatment in Australia and Europe.

In May 2014, Retrophin began a trial in Europe treating one patient with RE-024. The patient was unable to walk unassisted at the time and now walks freely. Which tells us that this drug is not only safe in animals but also in humans. Granted, this is one case, but when you have three boys with this deadly disease, one is enough for us. So with excitement comes more frustration, knowing that there is an opportunity to help our boys get better, but that we have an FDA that continues to deny us the only option available to us now.

Since April 2014, we have been lobbying to get our physician IND approved. We have met with four individuals at FDA headquarters in Maryland, met in Washington, DC with six United States Senators and 1 Congressman, of which all seven have been lobbying on our behalf in DC for approval. Our North Dakota Senators and Congressman have had two phone calls directly with FDA Commissioner, Margaret Hamburg, urging her to approve this IND. Commissioner Hamburg has the power to overturn the FDA review committee's decision with one phone call, yet we continue to be denied.

Other efforts have included the creation of an online petition urging the FDA to approve the IND along with time spent being interviewed on many local radio talk shows and local news stations. All of this has brought awareness to the issue but we still have not been able to proceed with the Physician's IND to treat the boys with RE-024. Time is of the essence, as each day they lose a bit more of their normal self. We are not asking the FDA to approve RE-024 for general use, only to allow it to be taken in a clinical trial by our children who are counting on this drug. It's shocking to me that we have a drug company willing to give us the drug, a doctor, who is seen as an expert on the disease, willing to administer it, parents willing to sign any documentation allowing treatment of our kids and boys who are begging for the drug so that they have a chance to live a normal life. Yet, individuals working from an office in Washington, DC, continue to deny treatment and tell us that they know better for our three terminally ill kids than their doctor, the drug company, their parents and the boys who are living with the effects of this disease daily.

I am urging you to support Senate Bill 2259. Several states have passed similar bills and it is raising awareness at the FDA that there is a need to revamp the current approval process for Investigational New Drug Trials. We are a country of great progress in the medical field, yet we sometimes fall behind other countries that are forward thinking in allowing new treatments in which the patient has no other option. Many families like ours are trying so desperately to get treatments to save their loved ones but the current bureaucratic processes at the FDA are leaving us with little hope. The need for change in expanded access needs to happen soon. Medical advances are on the rise and we will see more and more cases where a medication is available that could potentially help a terminally ill patient but they can't get through the red tape to save their own life. The time for change is now. We do not want anyone else to go through the same struggles that we have endured. We will continue to fight, not only for PKAN patients, but all patients that should have the right to try a drug when they have no other options.

As for our boys, Lane is having many physical troubles. Recently, he is loosing the ability to tie his shoes because his hands are starting to fail him. Writing in school is a challenge because of his hands. His speech is failing him also because of dystonia in his mouth, he is loosing the ability to communicate like he should be able to. He continues to fall down numerous times a day, on some occasions hitting his head. He needs RE-024!

Tanner is struggling with the use of his arms and hands. This was once a kid who was very active and in a short amount of time he lost the ability to do many everyday tasks. He can no longer dress himself, put shoes on, nor tie them. Because of his arms being spastic he is off balance and falls often. He also has dystonia in his mouth- speaking and eating is difficult. He also needs RE-024! As for all the boys, they take massive amounts of medicine daily trying to slow the progression of this disease and to help relax the spastic muscles in their bodies. Tanner takes the most pills, 27 pills daily! If we could have a chance at this new drug, we could eliminate all of them and take just one.

As a parent, one of the hardest things to watch is your kids struggling to do everyday tasks that they once could do easily. What may be worse, is listening to your children beg and plead daily for a medicine that we believe could reverse their symptoms and possibly take them back to a physical state that would allow them a normal life, once again. My boys have the same dreams as any other boy their age. They want to play football and basketball. They want to ride horse and show their animals at the state fair. They want to grow up and start families of their own. So many of those dreams have already been taken away from them, but they have an opportunity to achieve many of those dreams with your help. You can give them that chance. Without RE-024 those dreams will never happen. I beg you to support this legislation and give my family the hope that we are desperately seeking.

I thank you for your time today.

Laura Kulsrud
Grace City, ND
701.653.5930

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3/10/2015
page 1
#3

GOLDWATER INSTITUTE

Where freedom wins.

Facts About "Right To Try"

For terminal patients who have exhausted their conventional treatment options, obtaining access to potentially life-saving investigational medications is often extremely difficult. The patient can attempt to enroll in a clinical trial, but many of the sickest individuals do not qualify. In fact, only 3 percent of patients today are enrolled in clinical trials. For everyone else, their only hope for obtaining potentially life-saving medications is to ask the FDA for special permission.

Only about 1,000 people make it through the FDA's "compassionate use" application process each year. The process is complicated, time-consuming, and expensive. The first step in the process requires a doctor to complete an application that the FDA estimates takes 100 hours. After the doctor submits the application to the FDA, the manufacturer must also submit lengthy documentation requirements. The FDA then has a month to review the submission and either grant or deny the request, but if there are any questions the one-month clock starts over. After the FDA approves a request, a separate committee not affiliated with the FDA, called an Institutional Review Board, also must approve the patient's use of the drug. The Institutional Review Board can sometime take up to a month to reach a decision.

Sadly, there are many documented cases of patients dying while their application is being considered. The FDA recently announced plans to shorten the application, but the other steps will still remain in place. A shorter application for the first step is helpful, but it only addresses one part of the approval process. And ultimately, it's still an application to the government to ask permission to try to save your own life. If you have a terminal illness, you don't have time for a multi-step government process. If your child is dying from a terminal illness and you know there's an investigational medication that is already helping other children survive, a shorter form isn't good enough.

We need to remove barriers that limit doctors from providing the care they are trained to give—and this is exactly what Right To Try does.

Right To Try allows terminally ill Americans to try medicines that have passed Phase 1 of the FDA approval process but are not yet on pharmacy shelves. Right To Try expands access to potentially life-saving treatments years before patients would normally be able to access them.

Goldwater Institute
(602) 462-5000
www.goldwaterinstitute.org

Under Right To Try, a terminal patient would be able to access an investigational medicine if:

- ✓ The patient has a terminal disease and has exhausted all conventional treatment options;
- ✓ The patient's doctor has advised the use of an investigational medication;
- ✓ The medication has successfully completed basic safety testing and is part of the FDA's on-going approval process;
- ✓ The patient has provided "informed consent" acknowledging the potential risk of the drug; and
- ✓ The company developing the medication is willing to make it available to the patient.

Right To Try includes important protections. The basic safety testing and informed consent requirements protect the patient. And doctors and the manufacturer are protected from liability if the investigational medication doesn't work. But this is not protection from medical malpractice.

Right To Try is already law in Arizona, Colorado, Louisiana, Michigan and Missouri, where it passed with overwhelming bipartisan support. It has been introduced in 26 more states this year. Right To Try isn't a red or blue issue; it's a human dignity issue. That's why lawmakers from both sides of the aisle are coming together to give their citizens the Right To Try.

The FDA says providing dying people with investigational medications should be an exception. We think it should be the rule. People fighting for their lives should have access to medicines that could save them without needing a permission slip from the government.

For more information about Right To Try visit goldwaterinstitute.org. Or contact Kurt Altman, kaltman@goldwaterinstitute.org, (602) 462-5000.

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Page 1

#4

Testimony on SB 2259
2015 Legislative Session
March 10, 2015
Rep Weisz, House Human Services Chairperson

Representative Weisz and Members of the House Human Services Committee,

My name is Donene Feist, I am the Director for Family Voices of North Dakota. I come before you today to request support for SB 2259.

Family Voices of North Dakota is statewide health information and education center who serves families of children with special health care needs in ND. We provide emotional and informational support to many families across North Dakota who have a child who has an ongoing medical diagnosis such as asthma, heart conditions, terminal illness, physical disabilities, and emotional/behavioral issues. Our staff, who all are parents to children with special health care needs, provides assistance to families by helping them access and navigate services.

FVND has assisted families who face decisions each day that are very difficult decisions to make. When you have a child with a terminal illness you want to make the best decisions for your family and child. This is not done in isolation. Many families work diligently to receive the treatments needed for their child. Families cannot wait, expanded access to these necessary drugs are vital for families.

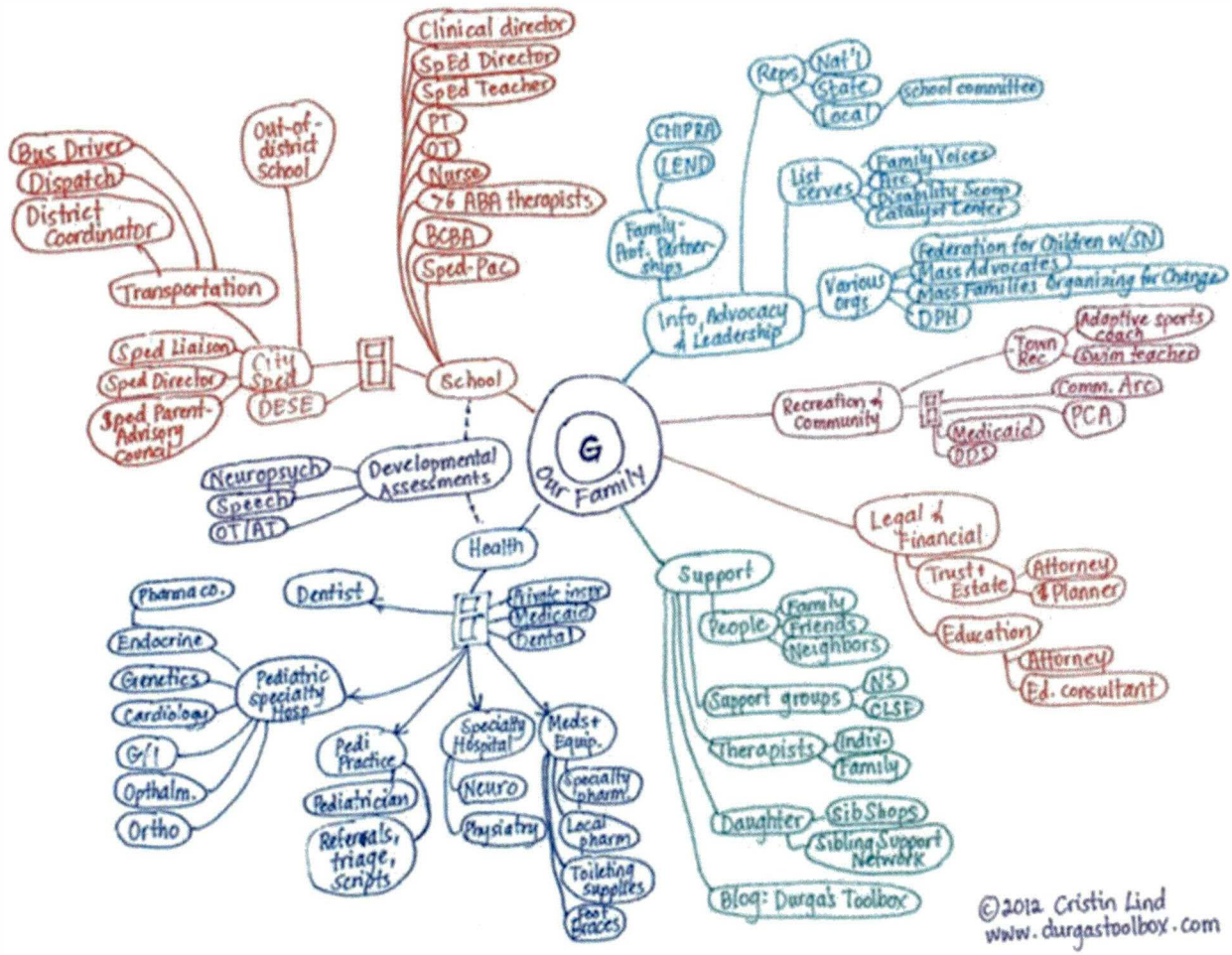
States have been leading the cause for change regarding Investigational Drug Trials in encouraging the FDA to change their policies regarding the approval process. My hope is that this committee will see the need to do the same.

In assisting other families across North Dakota, we know that the process and barriers to approving lifesaving medication is most difficult. Families should not have to wait and work through all of the barriers in order to assist their serious or terminally ill child. Often, there isn't the time.

We also know that through medical advances, there could be a medication available tomorrow. But with the barriers, families are still not able to participate. We can do better.

Not only will this bill help the children who face a terminal illness, it will also assist the adults. Individuals with cancer, ALS, MS to name a few. This is clearly something you can do as lawmakers to make a difference for the individuals and families who face a serious or terminal illness. This initiative would allow terminal patients access to investigational drugs that have completed basic safety testing, thereby dramatically reducing paperwork, wait times and bureaucracy, and, most importantly, potentially saving lives.

In the diagram below, I am leaving you with this visual of all of the services families encounter when they have a child with special health care needs. Families are left to navigate systems either on their own or with the help of an organization like FVND. As you can see from the diagram, many services are in silos. Having a child with a serious and terminal illness would be another layer of complexity that the family would need to endure.



The Goldwater Institute reports that while the FDA is tasked with protecting the public from unsafe and ineffective medications, the agency's approach is inappropriate in the context of terminally ill patients. The terminally ill face a much different risk-benefit analysis than the public at large. Patients who are not battling an immediately life-threatening illness are likely less risk-tolerant and more willing to wait for a proven cure, but terminal patients do not have the luxury of time. Many terminal patients who lack other treatment options may be willing, even eager, to try medications whose efficacy has not yet been established. Even the FDA has recognized that "for a person with a serious or life-threatening disease, who lacks a satisfactory therapy, a promising, but not yet fully evaluated product may represent the best available choice."[61]

As policymakers, what would you do if your grandchild or child had a serious/terminal illness that you knew there was a drug that may be able to assist? What if it was your spouse? I think we can all agree on what we would do. Changes can happen, you can make it happen.

You can send a message today to the FDA that changes need to happen.

It takes a family to raise a child, but it takes a village to support families so care can be coordinated.

In closing...."**Let us remember as each of us makes decisions that will affect children—whether we are parents, educators, health professionals, or government officials—it is our duty to consider if that decision either affirms or denies a child's most basic human rights.**"

States should enact "Right to Try" measures to protect the fundamental right of people to try to save their own lives.

Thank you for your consideration

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