2015 SENATE HUMAN SERVICES

SCR 4016

2015 SENATE STANDING COMMITTEE MINUTES

Human Services Committee
Red River Room, State Capitol

SCR 4016
2/9/2015
23447

☐ Subcommittee
☐ Conference Committee

Committee Clerk Signature Donald Mueller

Explanation or reason for introduction of bill/resolution:

A concurrent resolution urging Congress and the President of the United States to direct the federal Food and Drug Administration to allow the use of experimental medications to treat Pantothenate kinase-associated neurodegeneration (PKAN) for the benefit of the three children of the Kulsrud family living in Grace City, North Dakota.

Minutes:

Attach #1: Testimony by Sen. Heckaman
Attach #2: Written testimony by Laura Kulsrud
Attach #3: A Fight Against Time Article

Senator Joan Heckaman introduced SCR 4016 to the Senate Human Services Committee (attach #1). Senator Heckaman provided written testimony by **Laura Kulsrud** (attach #2) and a news article "A Fight Against Time" (attach #3).
4:29

Senator Dever asked if these are the only 3 children in North Dakota with this disease.

Senator Heckaman answer she was unsure, but did not believe there were others in North Dakota. Ms. Kulsrud had been on the internet and found several others with the same disease.

Chairman Judy Lee indicated that SB 2259 is related to this Senate Concurrent Resolution.

OPPOSITION TO SCR 4016
No opposing testimony

NEUTRAL TO SCR 4016
No neutral testimony

Closed Public Hearing

NOTE: Vote for SCR 4016 is under recording 23446 - SB 2259.

2015 SENATE STANDING COMMITTEE MINUTES

Human Services Committee
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SCR 4016
2/9/2015
23446

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Explanation or reason for introduction of bill/resolution:

A concurrent resolution urging Congress and the President of the United States to direct the federal Food and Drug Administration to allow the use of experimental medications to treat Pantothenate kinase-associated neurodegeneration (PKAN) for the benefit of the three children of the Kulsrud family living in Grace City, North Dakota.

Minutes: No Attachments

This vote is recorded under J#23446, concurrent with SB 2259.

Senator Warner moved that the Senate Human Services Committee DO PASS SCR 4016. The motion was seconded by **Senator Dever**. No Discussion

Roll Call Vote
6 Yes, 0 No, 0 Absent

Senator Warner will carry SCR 4016 to the floor.

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

2015 SENATE STANDING COMMITTEE
ROLL CALL VOTES
BILL/RESOLUTION NO. SCR 4016

Senate 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 Warner Seconded By Dever

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 Warner

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

REPORT OF STANDING COMMITTEE

SCR 4016: Human Services Committee (Sen. J. Lee, Chairman) recommends **DO PASS** (6 YEAS, 0 NAYS, 0 ABSENT AND NOT VOTING). SCR 4016 was placed on the Eleventh order on the calendar.

2015 HOUSE HUMAN SERVICES

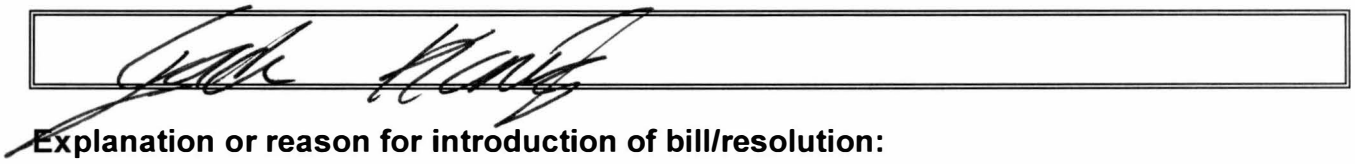
SCR 4016

2015 HOUSE STANDING COMMITTEE MINUTES

Human Services Committee
Fort Union Room, State Capitol

SCR 4016
3/17/2015
25009

☐ Subcommittee
☐ Conference Committee



Explanation or reason for introduction of bill/resolution:

Urging Congress and the President of the US to direct the federal FDA to all the use of experimental medications to treat Pantothenate kinase-associated neurodegeneration for the benefit of the three Kulsrud children.

Minutes: Attachment 1

Chairman Weisz: Opened the hearing on SCR 4016.

Sen. Joan Hermann: Introduced and supported the bill. (See Testimony #1)

NO OPPOSITION

Chairman Weisz: Closed the hearing on SCR 4016.

Chairman Weisz: What are the committee's wishes?

Rep. Porter: To me it does something a little bit more than just send the piece of paper, because I think when you have important issues such as this it is important to know that they got the message from us. I am not going to make the motion I am not going to do the amendment I am just going to say when it is at this level of importance that I think it is more than just a first class delivery situation. I move to adopt amendment that the copies of the resolution on line 1 of page 2 after the word resolution we insert "via certified mail return receipt requested"

Rep. Fehr: I second.

Motion to Adopt Amendment Page 2 line 1 insert after resolution "via certified mail return receipt requested"
Motion made by Representative Porter.
Seconded by Representative Fehr.
Voice Vote.
Motion Carried.

Rep. Oversen: Wondering if we might consider and additional therefor clause. Off the top of my head it would be "the 64th legislative assembly urges Congress and the President to direct the FDA to review their compassionate care application process so that more terminally ill patients may benefit from that process." I don't know the correct language off the top of my head. Part of the problem that we heard is that this process is really slow, only a small number of people are able to access it and that is a larger problem. If we want to keep this resolution clean I am fine with that as well.

Rep. Porter: I second

Rep. Becker: If we are going to do that shouldn't we have a time set, like do it immediately. Time is of the essence here.

Chairman Weisz: As far as her amendment she is looking at going forward in the future that the process should be looked at so I am not sure if you want to look at that as a time stamp. The resolution itself says the one child isn't expected to survive if something doesn't happen so I think that part says time is of the essence for that child. You have to look at the big picture and change the process of how you look at the passionate care for experimental drugs. Not sure we want to say you have to do it in six weeks or three months or whatever it might be.

Rep. Fehr: I am going to resists this amendment not because I disagree with it I am just thinking in terms of this particular resolution the amendment doesn't help this family and might even detract a little bit.

Motion to Adopt Amendment adding; the 64th legislative assembly urges Congress and the President to direct the FDA to review their compassionate care application process so that more terminally ill patients may benefit from that process.
Motion made by Representative Oversen.
Seconded by Representative Porter.
Voice Vote.
Motion Carried

Rep. Hofstad: I move a Do Pass As Amended and Place on Consent Calendar.

Rep. Seibel: Second.

Motion for a Do Pass As Amended and Place on Consent Calendar.
Motion made by Representative Hofstad.
Seconded by Rep Seibel.
Total yes 13. No 0. Absent 0.
Motion Carries.
Floor Assignment Representative Mooney.

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Title.02000

Adopted by the Human Services Committee

SJC 3/17/15

March 17, 2015

PROPOSED AMENDMENTS TO SENATE CONCURRENT RESOLUTION NO. 4016

Page 2, line 1, after "resolution" insert ", via certified mail return receipt requested,"

Page 2, line 3, replace the period with "; and"

Page 2, after line 3, insert:

"**BE IT FURTHER RESOLVED**, that the Sixty-fourth Legislative Assembly urges Congress and the President of the United States to direct the federal Food and Drug Administration to review and update the Compassionate Care application process so a greater number of terminally ill patients may benefit."

Renumber accordingly

Date: 3-17-15
Roll Call Vote #: 1

2015 HOUSE STANDING COMMITTEE
ROLL CALL VOTES
BILL/RESOLUTION NO. 4016

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. 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:

page 2, line 1 insert "via certified mail return receipt requested" after "resolve"

page 2, line 3 replace the period with "and"

Date: 3-17-15
Roll Call Vote #: 2

2015 HOUSE STANDING COMMITTEE
ROLL CALL VOTES
BILL/RESOLUTION NO. 4016

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. Oversen 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:

Instruct the FDA to review and update the Compassion Care application process.

Date: 3-17-15
Roll Call Vote #: 3

2015 HOUSE STANDING COMMITTEE
ROLL CALL VOTES
BILL/RESOLUTION NO. 4016

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 2

Floor Assignment Rep. Mooney

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

REPORT OF STANDING COMMITTEE

SCR 4016: 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). SCR 4016 was placed on the Sixth order on the calendar.

Page 2, line 1, after "resolution" insert ", via certified mail return receipt requested,"

Page 2, line 3, replace the period with "; and"

Page 2, after line 3, insert:

"**BE IT FURTHER RESOLVED**, that the Sixty-fourth Legislative Assembly urges Congress and the President of the United States to direct the federal Food and Drug Administration to review and update the Compassionate Care application process so a greater number of terminally ill patients may benefit."

Renumber accordingly

2015 TESTIMONY

SCR 4016

SCR 4016
02/09/15
Attach #1
Jtt 23447

SCR 4016

Chairman and members of the committee:

I am Senator Joan Heckaman from New Rockford and I represent District 23. I am here today to introduce SCR 4016 to you.

I am bringing the resolution after other attempts to help the Kulsrud family from Grace City, ND with access to an experimental drug to help their 3 children who have PKAN disease have been unsuccessful. My family grew up with Jay and have fond memories of being together with him and his family. In 2011 the family began a journey none of us ever want to be on. To make sure I have all of the information correct, I will read to testimony Laura Kulsrud e-mailed to me.

(e-mailed handout)

The resolution before you urges Congress and the President of the United State to direct the FDA to allow the family access to the experimental medication available to treat PKAN.

I urge you to pass this resolution and move this resolution along quickly as time is of the essence. As Laura stated, the congressional delegation have already become involved with no resolution at this point. I believe we have one more avenue and that is Congress and the President.

Please join me in passing this resolution.

Senator Joan Heckaman

J# 23447 SCR 4016, Attach #2 02/09/15

My name is Laura Kulsrud and I am the mom of 3 young boys that have been diagnosed with the deadly disease PKAN (Pantothenate kinase-associated neurodegeneration). My husband, Jay, and our three 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, balance problems leading him to stumble and fall often. After several tests Doctors diagnosed him with the disease PKAN. It is a genetic disease where a gene is mutated and then causes iron to accumulate in the basil ganglia of the brain, this then creates many neurological symptoms to occur. Since this was a genetic disease we had our other two boys tested. The test results came back positive that both Tanner and Ty also have the same disease but at the time had no symptoms. As you can imagine this was very devastating as there is no cure and our sons are going to die at an early age if a cure is not found. Since that diagnosis, Tanner is now experiencing some of the same problems Lane has. To this date, Ty remains symptom free, but he often wonders when this ugly disease will strike him. Ty is now at the age when the other two boys came down with the symptoms. Can you imagine the thoughts that go thru his head as he watches his brothers struggle each day?

We remained hopeful in the last few years that there would be a cure to get rid of this disease and our boys would be able to do what their peers are able to do. Over a year ago, we learned of a company called Retrophin that has been researching a new drug called RE-024 and have found that it cures PKAN when tested in animals.

RE-024 is a derivative of vitamin B5. People with PKAN cannot process vitamin B5 into phospho-B5. This then creates iron buildup and causes neuorological problems. When tested on rats and monkeys it 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. We were very hopeful that the boys would be allowed to start a trial of RE-024 until the hearing at the FDA denied the Physician's IND on April 10, 2014. We are very saddened by their decision to deny the medicine to our boys. Since May 2014, the drug has been used on a patient in Europe and has been showing success. The patient was unable to walk on his own and now is walking unassisted. Now we know that it is not only safe in animals but humans also. Can you imagine how excited we were to hear that RE-024 is working!

Since April 2014, we have been trying to lobby to get this physician IND approved for use in our boys. We have met with 4 individuals at the FDA headquarters, met with 6 different US Senators and 1 US Congressman, of which all 7 have been trying to get this approved. Our ND Senators and Congressman have also had two phone meetings with the FDA Commissioner Margaret Hamburg urging her to approve this IND, she was not willing to budge. She has the power to overturn the FDA review committee. We also had an online petition, talked on many local radio talk shows and local news stations. All this has brought awareness of PKAN but we still have not been able to proceed 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, only to allow it to be taken in a clinical trial by our children who are counting on this cure. We have a drug company willing to allow this drug to our children, a Dr who wants to administer it, we as parents want to see this happen, and children who want RE-024 so they have a chance at a normal life. I do now see a reason why the FDA should say no when we have nothing else to try.

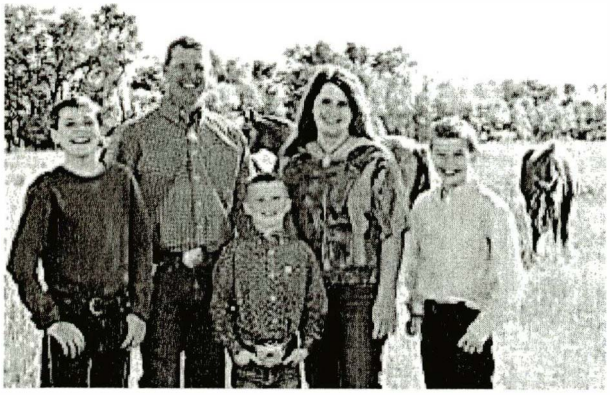
As a parent, one of the hardest things to watch is your kids struggling to do everyday tasks that they once could do. One of the other hardest things is listening to your children beg and plead daily for the medicine as they have many dreams of what they want to be when they grow up. Without RE-024 those dreams will not happen! Please help our family so that our three boys can be cured of this deadly disease!

Sincerely,
Laura Kulsrud
Grace City, ND
701.674-3400 home
701.653.5930 cell

Attach #3
02/09/15
SCR 4016
HB 23447

BROTHERS

A fight against time



MAY 03, 2014 6:00 AM • BY BRIAN GEHRING

GRACE CITY, N.D. — A Grace City family has taken the fight to save their three young sons from a rare and deadly disease to Washington, D.C.

Laura and Jay Kulsrud's three boys — Lane, 12, Tanner, 11, and Ty, 8 — have been diagnosed with PKAN, a progressive, degenerative nerve disorder caused by a mutant gene that allows iron to build up in the brain and usually results in death by early adulthood.

The family has twice been the recipient of trips through the North Dakota Make-A-Wish Foundation.

Lane was diagnosed with PKAN in December 2011 and Tanner has begun to experience the same symptoms as his older brother.

Ty, the youngest boy, has tested positive but has yet to exhibit symptoms, which include uncontrollable muscle contractions that can cause jerking or twisting, rigidity and stiffness of limbs, weakness and difficulty walking and talking, and slurred speech.

Laura Kulsrud said there is hope, though, through a new drug, RE-024, that has yet to be given to humans. And there is frustration.

She said the boys' doctor in Sioux Falls, South Dakota, petitioned the Food and Drug Administration more than a month ago for what is known as an IND, or investigative new drug application.

Federal law requires a drug be the subject of an approved marketing application before it is transported or distributed across state lines, according to the FDA website.

There are three IND types in two categories: commercial and research. The three types are investigator, submitted by a physician who both initiates and conducts the investigation; emergency, which allows the FDA to authorize use when time is critical; and treatment, which is for drugs showing promise while the final clinical work is conducted and the FDA review takes place.

3.2

Laura Kulsrud and Robin Anderson, a family friend, met with FDA officials Thursday and were told the FDA cannot approve a physician's IND or an emergency IND without a corporate IND being filed first.

Anderson, who works in marketing and consulting, said she was stunned at the FDA's response. She said the Kulsruds' doctor spent a month waiting for a decision on the physician's IND application and were told the commercial approval had to come first.

"Why spend 30 days working on a case ... when all along the only option was to deny it?" Anderson said.

Laura Kulsrud said the drug is manufactured by Retrophin, a New York company, and two weeks ago she and Anderson visited with company officials.

She said Retrophin officials told her that in tests on animals, the drug was shown to cure PKAN in rats and monkeys, even when given in high doses with no toxicity or other side effects.

Laura Kulsrud said there are two separate FDA groups that oversee the IND applications and each group gave different answers on the process.

She was told by one group that the application to enroll Lane and Tanner as part of a clinical trial was denied because there is insufficient data on the benefits versus the risks.

Laura Kulsrud said the benefit is the lives of her sons would be saved. The risk? "They are not going to die from the drug," she said. "They are are going to die from the disease."

PKAN typically strikes in childhood and is fatal 10-12 years after being diagnosed. Martin Shkreli, co-inventor of RE-024, said only about 1,000 kids in the U.S. and 6,000 worldwide have been diagnosed with the disease, which does not allow the body to process vitamin B5.

He said part of the problem is the FDA has no specific division to deal with what are known as "orphan diseases" — diseases that affect fewer that 200,000 patients.

Shkreli said orphan diseases are lumped into the same category as gastro-intestinal diseases and aren't given a high priority because there is a limited demand for the drugs used to treat them.

He said his company recently received a patent on RE-024. "We have been testing it vigorously," he said. "Ultimately, the drug has been proven to be effective."

Shkreli said in cases such as the Kulsruds,' the FDA must make exceptions.

"The rules need to change when lives are at stake," he said. "There is no greater risk ... they are on borrowed time."

Kulsrud and Anderson said meetings with FDA officials and Sen. John Hoeven, R-N.D., did provide a glimmer of hope.

Hoeven said he will follow up with the FDA in an attempt to expedite the process.

"That's what we're trying to work through," Hoeven said. "It's a serious situation ... we want the FDA to do everything they can to help this family."

In the meantime, Laura Kulsrud said every day that passes means more damage — at least for the two oldest boys.

She said Tanner especially has trouble with everyday tasks like getting dressed and eating. "Anything to do with his arms, really," she said.

Laura Kulsrud said she doesn't know offhand how much a trip to the drug store costs these days. "I don't even know. Tanner takes 27 pills a day," she said.

And while Ty hasn't started to show symptoms, he is at the age where he is starting to ask questions.

"He's scared," she said. "We all are. The boys just want to be normal.

"Every day we wait, there is more damage ... they don't get the fact that our kids are dying."

The boys have a Facebook page at https://www.facebook.com/kulsrudbrothers.

#1
SCR 4016
3-17-15

SCR 4016

Chairman and members of the committee:

I am Senator Joan Heckaman from New Rockford and I represent District 23. I am here today to introduce SCR 4016 to you.

I am bringing the resolution to help the Kulsrud family from Grace City, ND with access to an experimental drug to help their 3 children who have PKAN disease. In 2011 the family began a journey none of us ever want to be on.

Last week this committee heard SB 2259 designating North Dakota as a Right to Try State. I am not going to go through all of that testimony again because you can reference testimony from Laura Kulsrud and others at your convenience. One thing I want you to think about is the fact that the Phase 1 trial necessary before that bill can become effective for the Kulsrud children, may not come in time for all of the boys to take advantage of that piece of legislation or the medication. But this resolution may just be the opportunity the family has been waiting for if the FDA listens to Congress and the President of the United States.

The resolution before you urges Congress and the President of the United State to direct the FDA to allow the family access to the experimental medication available to treat PKAN. The family has already completed application to the FDA twice and has been rejected 2 times. The additional urging by Congress and the President may just be the factor that resolves this issue for the boys.

I urge you to pass this resolution and move this resolution along quickly as time is of the essence. As Laura stated in her testimony to the Senate Human Services Committee, North Dakota's Congressional delegation has already become involved with no resolution at this point. I believe we have one more avenue and that is Congress and the President.

Please join me in passing this resolution.

Senator Joan Heckaman