[LB91 LB401 LB481]

The Committee on Health and Human Services met at 1:30 p.m. on Thursday, February 2, 2017, in Room 1510 of the State Capitol, Lincoln, Nebraska, for the purpose of conducting a public hearing on LB91, LB401, and LB481. Senators present: Merv Riepe, Chairperson; Steve Erdman, Vice Chairperson; Sue Crawford; Sara Howard; Mark Kolterman; Lou Ann Linehan; and Matt Williams. Senators absent: None.

SENATOR RIEPE: (Recorder malfunction).

SENATOR HOWARD: (Recorder malfunction).

SENATOR ERDMAN: (Recorder malfunction).

SENATOR WILLIAMS: (Recorder malfunction).

SENATOR LINEHAN: (Recorder malfunction).

SENATOR RIEPE: (Recorder malfunction)...committee. Tyler Mahood is our committee clerk. Okay, and you'll be...if you're going forward, you'll have some interaction with Tyler over here. And now, Senator...

SENATOR CRAWFORD: Thank you. Good afternoon. Senator Sue Crawford, District 45.

SENATOR RIEPE: Thank you. And our other senator...

SENATOR KOLTERMAN: Senator Mark Kolterman, 24th District: Seward, York, and Polk Counties.

SENATOR RIEPE: Okay. Thank you all very much. We also have some pages that are working with us today and a great help: Brianne Hellstrom, who is from Simi Valley, California; I think...and also we have Jordan Snader from Oakland, Nebraska. We appreciate very much their being here. The committee today will be taking up bills in the order that they were posted. This is your opportunity to participate in the legislative process within Nebraska, and we very much encourage that. We will also have some committee members that, at times, will get up and go because they are either introducing other bills or they're testifying on other bills. All of the standing committees are holding committee hearings one day of the week or another, and so

we're always very busy. We also...you'll find that several members will be working on computers. We encourage that because all of the information that's on paper is now available on the computers. So they're not being distracted; they're being very attentive. Some of the rules of engagement, there's...please silence your phones, and we'll ask you, if you're testifying or opening, we will ask you to move up to the front to keep the process going. The process is: First of all, the individual who's introducing the bill will introduce it; followed by proponents; then we follow by opponents; and then we follow by any neutral; and if we have any letters that come in, we will read those letters; and then the introducer of the bill will have an opportunity to make the final and closing remarks. The testifiers are asked to sign in. We will have...ask you to hand in your orange sign-in sheets to the committee clerk when you come up to testify. We also, too, when you come up to testify, we ask you to state your name and then spell it out, and that's important for the transcribers. We work on a five-minute clock. For the...the introducer is unlimited time, but witnesses--we limit that to five minutes. It's four on the green, one on the amber, and then a red light will go on. And you don't have to abruptly quit at that moment in time, but we'll ask you to conclude and pull it together. And if it goes on, then I may ask you to try to pull it together and wrap it up. I do want to read this, too, and I want to make sure I get it into the record correctly. It says: if you will not, again, not be testifying at the microphone but want to go on record as having a position on a bill being heard today, there are white sign-in sheets at each entrance where you may leave your name and other pertinent information. These sign-in sheets will become exhibits in the permanent record at the end of today's hearing. Written materials may be distributed to committee members as exhibits only while testifying is being offered. Hand them to a page for distribution to the committee and staff when you come up to testify. We need ten copies for all of the members and the staff. With that, we will open the session. We are going to, today on our agenda, we are going to open with LB91, with Senator Hilkemann, and what we're doing is we're combining two of his bills: LB91 and LB401. So Senator Hilkemann, the mic is yours. [LB91 LB401]

SENATOR HILKEMANN: That's a two-for-one sale. [LB91 LB401]

SENATOR RIEPE: There you go. [LB91 LB401]

SENATOR HILKEMANN: (Exhibit 1) Good afternoon, Chairman Riepe and members of the Health and Human Services Committee. I am Robert Hilkemann; that's R-o-b-e-r-t H-i-l-k-e-m-a-n-n, and I proudly represent District 4 in the Nebraska Legislature. I am introducing, for your consideration, LB91 and LB401. Now LB91 would modernize terms in existing statute and add a definition for pharmaceutically-manufactured foods. LB91 also changes the fee that is set by the Department of Health and Human Services for the administration of the newborn screening program, to no more than $20. Now LB401 would add three diseases to the already existing newborn screening program. Those diseases are X-linked adrenoleukodystrophy, or X-ALD, mucopolysaccharidosis type 1, MPS 1, and Pompe disease. Now I could spend the entire

afternoon and tell you about those, but I've included them in that brochure and you can read about them, because I can tell you I knew nothing about these diseases until I brought this, until this was introduced to me. But believe me, they're...I can tell you that you do not want them nor do you want any of your family members to have them. You'll hear more about those. Today you will hear testimony from experts on these diseases, including doctors who have studied these diseases and family members who have experienced them. And I am neither of these experts. However, the story of how I came to be the introducer of these bills highlights the exact reason why I chose to become a state senator. In 2015 a constituent contacted my office and requested a meeting with me. Her name is Meghan, and you're going to hear from her in a little bit. We met for coffee and she told me her story about X-ALD, which she will be sharing with you today. Colleagues, when we run for the Legislature, it's the people like Meghan who we see ourselves coming to Lincoln to represent. Meghan isn't a lobbyist or a special interest group. She is a young woman who has experienced X-ALD in her life and wants to ensure that all family members have access to the life changing treatments available when an infant tests positive for these diseases. The newborn screening program in Nebraska is celebrating its 50th anniversary this year. It got its start in 1967 with testing of a condition that we all know, PKU, or phenylketonuria. When not diagnosed and treated, PKU causes severe developmental disabilities. But babies diagnosed and put on special formula in their first seven days can have life like everyone else. With the outstanding advancements in medicine over the last 50 years, Nebraska's newborn screening program now tests 29 different genetic and metabolic conditions. In 2015, 27,000 babies were screened in Nebraska, and 58 newborns were identified with conditions and treated in time to prevent or reduce problems associated with those conditions. That's 58 children who would otherwise be facing unimaginable challenges, hardships, and death at far too young of an age. It's also 58 families whose expenses for care would be exorbitant and could potentially fall to the responsibility of the state of Nebraska under the Medicaid program. You will hear success stories of children who have been benefited from the newborn screening program. Your handouts include one of those stories. When Tyler Morton was born, his newborn screening identified that he had a biotinidase deficiency. His parents were told that, if left untreated, Tyler would suffer from muscle weakness, developmental delays, breathing problems, hearing loss, vision problems, an inflammatory skin rash, hair loss, and, sometimes, seizures. By the time these problems show up, the damage may be irreversible. Thanks to the newborn screening program, Tyler was able to avoid these problems with the addition of high doses of biotin. The treatment is easy, inexpensive, and effective. You'll also hear stories that tell you what can happen without the time imperative in identifying these conditions. Unfortunately, Bob Rauner, who many of you have met, cannot be here today to tell you his story. Thank you to those of you who took time, over the last few weeks, to meet with Bob. His written testimony has been provided to you, but I want to make sure that a little bit of his story is told today. Bob is the parent of children who have been affected by ALD. Imagine your young child showing a reversal in his educational development, having difficulty interacting with his fellow classmates, and also having vision problems. You take him to the doctor and you're told that your child has ADHD, so

for years you treat him for attention deficit hyperactivity disorder, but it continues to get worse and decline. Then, when he is 11 years old, you're told that diagnosis was wrong, and your child actually has ALD, and he would have a year to live. That is Kevin's story, one of Bob's sons. Paul is another of Bob's sons. Paul is affected by the adult form of the disease, which is called AMN. Paul now uses a wheelchair for mobility, and the disease has also affected his ability to continue at his job. One family, two sons. Bob's quest to find anything that could help save his sons led him to his current role as president of the United Leukodystrophy Foundation. There was no cure, nor were there effective treatments for Kevin or Paul. There wasn't newborn screening for ALD at the time they were born, but it is available now. It is available and it is a fraction of the cost that families, insurance companies, or the state of Nebraska would have to spend for even one baby born with ALD. Bob's written testimony includes compelling information about the statistics of ALD and how early intervention can prevent seemingly healthy children from an adrenal crisis that can result in their sudden death. I admire Bob because he isn't working for himself or for Kevin or for Paul. He is working for the families who are bringing beautiful babies into this world who have a 1-in-17,000 chance of having ALD, a disease that we can keep from taking the lives of those precious children. For every story about ALD, there's a story about MPS 1 and also about Pompe. You'll hear some of those stories today. They are all important. At the national level these three diseases have been added to the recommended uniform screening panel by the United States Secretary of Health and Human Services. At the state level, it has been recommended to the Department of Health and Human Services by the Nebraska Newborn Screening Advisory Committee. Dr. William Rizzo, a pediatric genetic and metabolic specialist, is a member of that committee, and he is here today to speak to the importance of adding these diseases to the newborn screening panel. The committee clerk provided you with a letter from Dr. James Harper, and it is included in my handouts, as well. Dr. Harper proposes a change in the method of funding the program, which would remove the now $10 or proposed $20 limit and allow for the Department of Health and Human Services to account for the cost of the program on a biannual basis, with the cost prorated per newborn. He suggests the possibility that the newborn screening program can become self-funding. I am intrigued by that possibility, but I do not feel that an amendment to LB91 to accomplish this would be prudent at this point. I would, however, like to encourage the committee to consider an interim study on that particular matter. The newborn screening program is an investment in healthy Nebraska babies. The costs upfront to administer the program are a reality, but it's a no-brainer when you consider the long-term cost savings to families, insurance companies, and the state of Nebraska's Medicaid program. Thank you for your consideration of these bills today, and I look forward to a great discussion and working with the committee to advance them to General File. Affectionately...when you...you will hear from Meghan in a few minutes, and I have affectionately called this the Nebraska Meghan's Law. So with that I will conclude my opening testimony and answer any questions you may have. [LB91 LB401]

SENATOR RIEPE: Thank you, Senator Hilkemann. For those of you in the room who don't know this, Senator Hilkemann is also Dr. Hilkemann, a physician. So that gives him a little bit of an advantage on knowledge. So we thank you for your presentation. [LB91 LB401]

SENATOR HILKEMANN: You're welcome. [LB91 LB401]

SENATOR RIEPE: We're now going to ask and see if our committee members have some questions. [LB91 LB401]

SENATOR HILKEMANN: Okay. [LB91 LB401]

SENATOR RIEPE: Senator Williams. [LB91 LB401]

SENATOR WILLIAMS: Thank you, Senator Riepe. And thank you, Senator Hilkemann, for being here and bringing that. And I am glad you are a doctor, because you can pronounce those things and the rest of us can't (laughter). I have a question about your introducing both LB401 and LB91. [LB91 LB401]

SENATOR HILKEMANN: Right. [LB91 LB401]

SENATOR WILLIAMS: Are you looking at combining those two as... [LB91 LB401]

SENATOR HILKEMANN: Yes. The LB91 basically was some clean-up work that they needed to have done for the newborn screening exam. And we incorporated it into this business. [LB91 LB401]

SENATOR WILLIAMS: Then can you take me through so we are very clear, at least I am very clear, on the understanding that you're...we would be adding these 3 new screenings to the 29 that are there. [LB91 LB401]

SENATOR HILKEMANN: That's correct. [LB91 LB401]

SENATOR WILLIAMS: And there is a fiscal note. It looks like that it is designed to...so that the department can create the information that would be necessary. Is that what's happening with the fiscal note? [LB91 LB401]

SENATOR HILKEMANN: That's exactly correct. And the dissemination of that information, that's correct. [LB91 LB401]

SENATOR WILLIAMS: Correct. Now tell me how these screenings get paid for now and how they would get paid for following the implementation of your legislation. [LB91 LB401]

SENATOR HILKEMANN: Okay. Well, they would be just as any normal child is...we take that test and it's added to the cost of their hospital bill. And it's paid for by insurance companies, in most instances. [LB91 LB401]

SENATOR WILLIAMS: And it's limited to no more than $20? Is that... [LB91 LB401]

SENATOR HILKEMANN: I think this one is $23 or something like that, right. [LB91 LB401]

SENATOR WILLIAMS: Okay, thank you. [LB91 LB401]

SENATOR HILKEMANN: Okay. [LB91 LB401]

SENATOR RIEPE: Okay. Senator Erdman. [LB91 LB401]

SENATOR ERDMAN: Thank you, Senator Riepe. Senator Hilkemann, help me understand what is the difference between a disease and an inherited defect? Some of these are inherited. Is that correct? [LB91 LB401]

SENATOR HILKEMANN: That's right. [LB91 LB401]

SENATOR ERDMAN: So can you explain those? I am not a doctor nor pretend to be, so help me understand that. [LB91 LB401]

SENATOR HILKEMANN: So let me help. I need to explain. You're seeing it...these are generally...these are genetic diseases... [LB91 LB401]

SENATOR ERDMAN: Okay. [LB91 LB401]

SENATOR HILKEMANN: ...that we can...that there's screening that can be done through chromatography that will show the different protein levels and so forth. There's a different

test...they're sort of tested each. But yet we can still put them all together in one nice vial and get them all done at one time. [LB91 LB401]

SENATOR ERDMAN: Okay. [LB91 LB401]

SENATOR HILKEMANN: For the little child, it's a matter of sticking the bottom of their heel. [LB91 LB401]

SENATOR ERDMAN: I understand that. I've had several calls from people in my district that wanted to know if there would be an option to opt out of this. Is that something that they need to have? And I am not sure they understand the deal. That's why I wanted to ask you that. [LB91 LB401]

SENATOR HILKEMANN: Yeah, I don't...yeah, I am not so sure you...this is not like a vaccination. This is to...this is routinely done on every child that's born. I mean you want to...these are just being added to the...there's...I think we have a list of all the tests that are part of the 29 that are presently being done at the present time. [LB91 LB401]

SENATOR ERDMAN: And I tried to listen to your testimony as Senator Chambers tells us to listen. And I think you alluded to there's 27,000 births. Is that all the births there were in the state? You say 27,000--something. [LB91 LB401]

SENATOR HILKEMANN: Yes, within Nebraska. Right. [LB91 LB401]

SENATOR ERDMAN: Okay, thank you. [LB91 LB401]

SENATOR RIEPE: Any additional questions? Okay, we will wait for your closing. [LB91 LB401]

SENATOR HILKEMANN: Okay, thank you. [LB91 LB401]

SENATOR RIEPE: And we will take proponents. Welcome. [LB91 LB401]

DR. WILLIAM RIZZO: Thank you. Senators, my name is Dr. William Rizzo. I am a professor of pediatrics at UNMC, and I treat patients with metabolic diseases, such as we're talking about. [LB91 LB401]

SENATOR RIEPE: Would you be kind enough to spell your name, please. [LB91 LB401]

DR. WILLIAM RIZZO: William Rizzo, R-i-z-z-o. And as you know, every child born in Nebraska is screened for certain metabolic diseases that are on the screening panel, the 29 diseases that we've already heard about. These diseases are screened because they simply cannot be detected at birth. And what happens is these children develop symptoms later on, usually in the first or second or later in life even, many of them in the first few weeks, or even few months, of life. They're difficult to diagnose, they're rare diseases individually, and so physicians have a hard time coming up with the correct diagnosis and therapy for these children. We have in Nebraska, as you mentioned, 27,000 births. Altogether about 1 in 1,500 will have a metabolic disease and, by that, as you've heard, these are genetic, inherited diseases. Parents are usually carriers for these diseases, but they don't know that they're even running in the family, in most cases. Those parents take home a baby that they think is healthy until something goes wrong. And it can go wrong in the first few months or the first few years of life or even, sometimes, later in childhood than that. We have a wonderful system in place here, newborn screening. Most people don't even know about it because it's so transparent, or hidden I should say--maybe that's a better word. They take their baby home, they have the heel stick blood sample taken. And almost all the time, with the exceptions that I mentioned, it comes back normal and they don't even know about it. It's a wonderful system. It's probably one of the best public health systems around. If you look at the productivity of it, the outcome of it, it saved lives. And frankly, this bill--these two bills together--will save lives. We will have children born over the coming year with one or more of these diseases. And frankly, we've seen this all too often. I am faced then with a child who has advanced in the disease to the point where we can't do as much as we otherwise would do. The reason for doing the screening is to diagnose them before they develop symptoms. And once we know that they have that disease, they've inherited that disease, then we can monitor them and treat them appropriately before anything serious happens. And you'll hear today about some therapies, that are ongoing for these diseases, that are quite effective. There are three diseases we're adding to the panel. X-linked adrenaleukodystrophy is a disorder that affects males predominately, and they don't develop symptoms until they're maybe five or six years of age, or seven or eight. We can do things for that if we know that they have problems right from the start. So we have a test that has now been advanced, so we have the technology to detect this disease at birth. We have a handful of these children that we follow in Nebraska, that I take care of. We do special tests, for example, brain MRIs to detect any evidence of neurologic disease before they even develop the symptoms. And at that point, we can treat them appropriately. The other disease, mukopolysaccharidosis type 1, is a disease that also is inherited, and those children look normal at birth. But sometimes, during the first year of life usually, they start to develop problems with liver and with heart. And they show changes in their facial structures, okay, over time. And then the diagnosis is made, hopefully; many times it's not made until it's gone on for several years even. But if we catch them early enough, like this, at the time of birth, we can diagnose; then we can treat them appropriately and prevent some of the serious and life-

threatening problems. The third disease is Pompe disease. These children are born. Sometimes they have no symptoms whatsoever, but sometimes, in the first few months of life, maybe the first year of life, they develop heart disease, congestive failure. And they die from the disease, usually before the year ends--scariest disease. We now have therapy for that, as you will hear today. We can prevent some of these serious complications of the disease. And children survive in ways that they never did before. These are the three diseases we're talking about. We will certainly want to add these to the panel, as recommended at the federal level. States now are starting to do that around the United States. We will hopefully be one of those. And when we do that, we'll be able to certainly have a major impact on families throughout the state. I am happy to take any questions at this point. [LB91 LB401]

SENATOR RIEPE: Thank you very much. For me, would you repeat your medical subspecialty? [LB91 LB401]

DR. WILLIAM RIZZO: Yes, I am a geneticist, a medical geneticist with the expertise in metabolic diseases, inherited metabolic diseases. [LB91 LB401]

SENATOR RIEPE: Okay, thank you very much. Are there questions of the committee? I have a question. My question would be, is this is 2017; why now? Why not five years ago? Why not... [LB91 LB401]

DR. WILLIAM RIZZO: We didn't have the technology five years ago. [LB91 LB401]

SENATOR RIEPE: Okay. [LB91 LB401]

DR. WILLIAM RIZZO: We now have it, and it can be added on to the screening card of the blood spots that are taken at the time of birth. It doesn't add any more blood samples from the child; it's just done, you know, as an add-on to the tests that are currently done. [LB91 LB401]

SENATOR RIEPE: Does this replace some others because, coming from a hospital background as well, I know that some of the testing devices can go up to 27 or whatever. Is this... [LB91 LB401]

DR. WILLIAM RIZZO: This is a really... [LB91 LB401]

SENATOR RIEPE: This doesn't require a replacement of capital at the hospital level is what I am saying. [LB91 LB401]

DR. WILLIAM RIZZO: No, no. We have a contracted state screening laboratory that does all the tests for us. That laboratory has been very efficient. We've had them for about ten years now. That laboratory is based out of Pennsylvania. Samples are taken from infants here, they're shipped overnight to Pennsylvania, that lab...that lab does all the testing and gives us the results. [LB91 LB401]

SENATOR RIEPE: Okay. [LB91 LB401]

DR. WILLIAM RIZZO: I might add we have one of the most outstanding newborn screening programs in the country. It has been recognized nationally. We're very active; we're very good at this. We need to keep up to pace with all the advances that are happening, as other states are doing now. [LB91 LB401]

SENATOR RIEPE: Okay. Erdman, Senator Erdman. [LB91 LB401]

SENATOR ERDMAN: Thank you, Senator Riepe. Thank you, Doctor, for coming. I have several people that have contacted my office who give birth at home. How would those people be handled? Or how are they handled now? What kind of screening do those people get? [LB91 LB401]

DR. WILLIAM RIZZO: Yeah, that's the good question. I'd like to address that, as well as the question about opting out. As you may or may not know, Nebraska has a law to require newborn screening of all infants--all infants. You cannot opt out of the screening. [LB91 LB401]

SENATOR ERDMAN: Okay. [LB91 LB401]

DR. WILLIAM RIZZO: Bills...or cases have come to court. They've gone to the Supreme Court, and they have upheld that law. So we have a very active, very good program here among our state employees in this newborn screening program, where they will keep track of every single birth, home births as well as births in the hospital. And we screen all the home births, as well. [LB91 LB401]

SENATOR ERDMAN: Okay. [LB91 LB401]

DR. WILLIAM RIZZO: The midwives that deliver at home recognize that this is a requirement and has been for many years. [LB91 LB401]

SENATOR ERDMAN: Okay, thank you. [LB91 LB401]

SENATOR RIEPE: Other additional questions? Concerns? Okay, thank you very much for coming. [LB91 LB401]

DR. WILLIAM RIZZO: I can address, I could address one other question... [LB91 LB401]

SENATOR RIEPE: Please do. [LB91 LB401]

DR. WILLIAM RIZZO: ...that was raised with Senator Hilkemann, if you wish. [LB91 LB401]

SENATOR RIEPE: Please do. [LB91 LB401]

DR. WILLIAM RIZZO: The cost of the test...we have a contract with the laboratory. They charge us a certain amount. The state has a fee of $10 tagged on to that so we can run the program. I've been here for 15 years now. That fee is the same; it has not changed. When I arrived here, we were screening for 6 diseases; now we're doing 29. We need a lot of help in the state program there to follow up on all these new diseases that are being picked up. Our employees are among the best I've ever worked with. And I think that this is certainly a great need, as I see it, for our state as well as our country, for that matter. You asked about the fee to the hospitals. So that fee, the cost that's bundled in, it's about $33, $34; I don't know the exact number myself. That's passed on to the hospital, the hospital tags on their own fee to collect the sample and to ship it out to the screening lab. So there is a cost that's beyond that, but it's bundled into the hospital bills and charges. And each hospital sets their own fee for that portion of the bill that's made. So one hospital it may be a total of--I don't know--maybe $60, another hospital it may be $100. But it varies from hospital to hospital in that regard. We have no control over that. [LB91 LB401]

SENATOR RIEPE: Okay, that's been very helpful. Thank you very much. Senator Crawford. [LB91 LB401]

SENATOR CRAWFORD: Thank you, Senator Riepe. And thank you, Doctor. So just to clarify, currently there's a $10 fee attached to that price to help pay for the cost to the state of managing this information and staff that's required. Is that what I heard you say? [LB91 LB401]

DR. WILLIAM RIZZO: That's correct. Now there is...there is funding for the program beyond that. Yes. [LB91 LB401]

SENATOR CRAWFORD: Okay, I was just asking because the fiscal note noted a need for general funds. And so I was just checking to see... [LB91 LB401]

DR. WILLIAM RIZZO: Yeah. [LB91 LB401]

SENATOR CRAWFORD: There is a fee that could perhaps pay for part of that, or we could revisit that question. Thank you. [LB91 LB401]

DR. WILLIAM RIZZO: And that fee is passed on to the insurance companies. [LB91 LB401]

SENATOR CRAWFORD: Okay, thank you. [LB91 LB401]

SENATOR RIEPE: So would the $10 offset the fiscal note or the fiscal note exists on top of the $10? [LB91 LB401]

DR. WILLIAM RIZZO: I am not able to address that. [LB91 LB401]

SENATOR RIEPE: Okay. Okay, we'll find out as we go along. Thank you again very much. Additional proponents, please come forward. [LB91 LB401]

MEGHAN KORTH: All right, good afternoon. Thank you guys for having me today. My name is Meghan Korth, M-e-g-h-a-n K-o-r-t-h. And my father, Paul Korth, passed away on January 25, 2015, just two years ago, as of last week. My dad was the kind of dad that didn't need to raise his voice to be heard. Some things we knew for sure, and they were that we were loved beyond words. And on good, bad, and even very ugly days, we did not question the pride he took in just being our dad. He didn't miss a game, and it was everyone's favorite guy to sit by in the stands, as he was just as quick to clip the newspaper articles, after a big game, for their kid as he was for his own. I cannot count the number of times in the past two years that someone in my life has come to me with a problem to be solved, and I've thought: oh, my dad can for sure help you with that. And, just as quickly I am forced to will my heart back together and make the correction: my dad could have helped you with that. While his absence is what's trying to take center stage, what is most significant about his story is that it was cut much too short. He wasn't supposed to be done here yet, but X-ALD had another idea, and that is the reason I am here today. My father is 1 of 10 children, 7 boys and 3 girls. Passing away at the age of 52, my dad outlived half of his brothers, as 3 died before they reached the age of 10 from X-ALD. My dad grew up asymptomatic and active, always involved in something. He went to college, married my mother, and they started their family. When I was 2 years old, an everyday routine became disrupted, as my dad started to find himself tripping when he would take a run around our house. Through

several doctor visits, much exploration, and many dead ends, they found the diagnosis--adrenomyeloneuropathy, which is the adult form of X-ALD. Momentarily this diagnosis halted my parents in their tracks, as it came with only one outcome. The lingering questions for the young parents of a two-year-old and one on the way were how long he had to live and what his quality of life would look like. And those were just dead ends, as AMN and X-ALD operate under their own agenda. However, within this diagnosis were answers, answers for his extended family and an opportunity for the next generation to not have to live through the same horror they were about to, the same opportunity Nebraska could give families through adding X-ALD to the newborn screening panel. This would mean the chance for a bone marrow transplant to allow some Nebraska boys to spend their elementary years on a baseball diamond and in the classroom, instead of in a hospital bed with hope out of reach. It would mean less Nebraska women watching the death of one family member and shouldering the reality that it could be relived within their future families. Truly what a privilege it is to sit before you, a privilege I loathe, as the only reason I am here today is because my favorite person is not. The two years preceding my dad's passing are the hardest to relive, but paint a picture so well of an outcome preventable for other families, preventable only through newborn screening. My dad was an active member of his community, his family, and his workplace. And it felt like overnight that was all forced to change. And when we woke up, he was no longer able to walk, talk, eat, communicate, or move on his own. The disease moved slowly and then all at once. And all at once our privileges of being his wife and daughters dramatically reversed roles. The man who always cared for us, guarded us, and raised us up was no longer able to be the man he deserved to be. And now we got to feed, bathe, give meds, change, lift, move, entertain, and love our favorite person 24 hours a day, seven days a week, for the two years he spent on hospice in our home. And this undertaking was our greatest honor. I was able to share a story at his funeral that highlights the man he was and the light heart he kept during the most devastating years of his life. Right before he went on hospice, he was bound to a wheelchair while also losing his ability to speak and eat. We were spending the evening with a group of basketball families, for appetizers and conversation, both of which were major obstacles for him. Throughout the night, I would step away from my friends to see how he was doing. "Can I get you anything to eat, Dad?" I asked. To my surprise, he nodded back yes, the only way he was still able to communicate. But now I had to deliver. I scanned the food table and made my offers to him, according to the least amount of mess that would be sure to ensue. "How about some veggies?" Quickly shut down. "Cheese and crackers?" That was another no. "How about something to drink?" Nope. With every shake of his head, his smile grew bigger; the guy knew what he wanted. As I scanned the table for what he could possibly want, I found it, and this time it was my head that was the one shaking no. "Dad, that cupcake...is that what you want?" He didn't even have to answer, as his face was lit up. This cupcake was large, it was all chocolate, and it had probably three inches of rainbow frosting on top. And we were not in the comfort of our own home, to enjoy it without attracting a copious amount of attention. But he knew he had me roped in. That night, among family and friends, my dad consumed as much of that cupcake as possible,

that is, of course, what was not a mess all over both of us or the floor. That bit of cupcake would be the last he would ever be able to eat. Life is unexpected, and it's hard. No one is exempt from its ruthless realities. My desire, in coming here today, is that this is the last time you hear this story, in the state of Nebraska, on account of X-ALD. This matter is not limited to allowing boys to live with less restrictions or giving families access to more programming. We are at the heart of whether some children will have life at all or if they will be taken down, at their most vulnerable, by a disease we could have alerted them about. As a human body, we long to rid the world of what devastates our families, and we are constantly let down as we cannot prevent every life-threatening condition. In contrast, we have the great privilege and responsibility to change the outcomes that fall within our power. And today we have that power. I urge you to consider, with great care, LB401, and not with the sense of wonder or curiosity, but with a conviction of the life that it will provide your community, your family, and the great state you represent. Thank you so much for your time. [LB91 LB401]

SENATOR RIEPE: Thank you very much. Are there questions from the committee members? Seeing none, thank you very much. [LB91 LB401]

MEGHAN KORTH: Thank you. [LB91 LB401]

SENATOR RIEPE: Our next proponent, please. [LB91 LB401]

JEFF SWANSON: Thank you for having me. My name is Jeff Swanson, J-e-f-f S-w-a-n-s-o-n. I live in Elkhorn with my wife and my daughter, Brooke, who has MPS 1, or Hurler Syndrome. It's named after the doctor that diagnosed it in Germany. MPS 1 is a storage disease, which means the simple sugars that break down, as you progress, normally dispose in your waste stream. And she does not possess the enzyme that helps these break down and induce, so it's a storage. And the waste buildup affects almost every symptom, or every portion, in your body. Without treatment, the general effect is that the child will possibly live until seven. My daughter...I guess we were lucky. She had other medical issues. So she was diagnosed at four, because we had a radiologist at Children's that recognized a unique shape in her ribs. So we were able to see a bone marrow transplant at about nine months. It being a progressive disease, the earlier you can get it, as Dr. Rizzo had said, and he's seen my daughter, the more these things can happen. And now that enzyme replacement therapy for this also...that is generally done in conjunction with the bone marrow transplant. And as the science continues to improve, the outcome of these children's lives continues to improve. So overall, I just wanted to thank you for having me here, and I wanted to do what my part is to maybe explain to you guys how this can help with a family member and a child's life. So do you have any questions for me? [LB91 LB401]

SENATOR RIEPE: Thank you for sharing that. Are there members from the committee? Senator Howard. [LB91 LB401]

SENATOR HOWARD: Thank you, Senator Riepe. How old is your daughter? [LB91 LB401]

JEFF SWANSON: She turned 18 in September. [LB91 LB401]

SENATOR HOWARD: 18? What's she up to now? [LB91 LB401]

JEFF SWANSON: She's going to high school at Elkhorn. [LB91 LB401]

SENATOR HOWARD: Oh, that's wonderful. Is she looking at colleges? [LB91 LB401]

JEFF SWANSON: We are working through that with voc. rehab and stuff like that...whether we can go do that or some work processes and stuff. [LB91 LB401]

SENATOR HOWARD: Well, we're very grateful that you shared your testimony today; thank you. [LB91 LB401]

JEFF SWANSON: Thank you. [LB91 LB401]

SENATOR RIEPE: Thank you very much. Additional proponents. [LB91 LB401]

SHANNON BUTALLA: (Exhibit 2) Good afternoon. I am Shannon Butalla; that's S-h-a-n-n-o-n. My last name is B-u-t-a-l-l-a. Our journey to understanding the magnitude and devastation of peroxisomal disorders began in November 2002, when our son, Sam, was diagnosed with a Zellweger spectrum disorder which you haven't yet heard about today. In 2002 Sam was born at full term, seven pounds, nine ounces. My pregnancy was rather uneventful except for an ultrasound that detected that Sam's waist measurement was smaller than expected, suggesting failure to thrive in utero. With a more advanced ultrasound, it was detected to be within normal range, and I continued to carry Sam to full term. Childbirth was difficult. Sam delivered posterior, or sunny side up. Suction was used to assist his arrival into the world, which we now know was due to his lower-than-normal muscle tone. Some of his blood work came back abnormal, and his breathing was labored after delivery. Everything resolved rather quickly; however, I could not help but notice Sam's large soft spot in his head, his skinny legs, and a weak suck when feeding. The labor and delivery nurses dismissed my concerns, and in my post-delivery fog, we went home as scheduled. The next four months were hell. Sam tested positive

for salmonella after we left the hospital that they believe he contracted from a fecal oral exposure there. He had as many as 26 stools a day. Our primary care physician bounced us between his office and the hospital lab for weight checks, blood draws, and stools--stool samples. Sam was not getting better and, although he wasn't losing, he wasn't gaining weight as he should. Sam's ongoing health issues led us to many specialists. We started with infectious disease, then neurology, then hepatology and genetics. The hepatologist thought Sam may have biliary atresia, where the bile ducts are blocked or absent, which would be fatal if untreated. A liver biopsy showed otherwise, so the journey towards diagnosis continued. November 19, at four and a half months of age, we received Sam's diagnosis: peroxisomal biogenesis disorder in the Zellweger spectrum of disorders, PBD-ZSD. He would be deaf, blind, cognitively disabled, physically disabled, and would not grow and thrive like a typically developing child. Sam's hepatologist told us many children with the disease didn't live to be one, and most died during their toddler years. Over the next months, we were flooded with medical appointments. Sam's vision and hearing were deteriorating. Sam began extensive physical and occupational therapy. He was fitted with hearing aids. We traveled to the Kennedy Krieger Institute at Johns Hopkins to meet with a world-renowned specialist on the disease. There we learned a specialist new to Omaha could provide the symptomatic and supportive care that we needed, Bill Rizzo. Sam has lots to say, too (laughter). [LB91 LB401]

SENATOR CRAWFORD: Yeah. [LB91 LB401]

SHANNON BUTALLA: These physician/scientists offered us a different picture and gave us hope. Although Sam would never be like his typically developing peers, he would write his own story and progress at his own pace. The disease would shorten his life, but there were no absolutes. Sam thrived beyond expectations. He learned to walk, swim, eat orally, and communicate through sign. It was slow, but he progressed. At age six, he experienced another hurdle no other child with PBD had faced: acute lymphoblastic leukemia. As a parent of a child who received a potentially fatal diagnosis as an infant, I am different of parents of boys with X-linked ALD. Sam never presented as normal, and symptoms from the disease were never...were apparent soon aster birth. There are similarities, however, in that Sam further declined when leukodystrophy, or white matter disease, presented. This wasn't until Sam was nearly seven and a half, around the time that some boys with X-linked ALD begin to show symptoms of their disease. Boys with ALD present normally until, sometime in their childhood, they exhibit escalating behavior, symptoms of ADHD, clumsiness, difficulty with handwriting, and gait issues. Unless there is a family history, there's nothing that would prompt families to test for such disease. By the time symptoms present, much of the damage is done. I can empathize with these boys' families, watching their child decline. In six short months, amidst heavy doses of chemotherapy for Sam's leukemia, he developed this leukodystrophy. All the skills Sam had worked so hard to obtain were gone. He went from walking to crawling, to "scootching," to having no independent mobility. He developed hemiparesis in his hand, tremors, inability to

balance, and rigidity that didn't allow him to function physically, as he had just six months before. It was, and still is, devastating for all who know and love him. The newborn screening proposed in LB401 would allow families with an X-linked ALD diagnosis to...before generative defects present. There are early treatments available, including the treatment of adrenal dysfunction, that prevents death and morbidity due to Addison's Disease. Those identified can be monitored, between the ages of two and ten, for early brain disease through MRI, that can be successfully with bone marrow or gene therapy treatments. The newborn screening also detects three other diseases, including peroxisomal biogenesis disorders, like Sam, and two other less common peroxisomal fatty oxidation disorders. Needless to say, having a child who is the statistic makes LB401 very important to my family and me. Although there are currently no effective treatments for Sam or children with his disease, it is thought that there...the children born with PBD-ZSD that are never diagnosed. Knowing these individuals, whether they present mild or severe, is the window to greater understanding of the disease and, one day, could lead to potential treatments. For boys with ALD, there are treatments, and it is our duty to protect them and their families from the debilitation and devastation that accompanies the diagnosis. Thank you for your time and consideration of LB401. [LB91 LB401]

SENATOR RIEPE: Thank you for presenting. Let's...are there questions from the committee? Thank you very much. [LB91 LB401]

SHANNON BUTALLA: Thank you. [LB91 LB401]

SENATOR RIEPE: And additional proponents. [LB91 LB401]

ASHLEE SPRINGER: Hi. I am Ashlee Springer, A-s-h-l-e-e S-p-r-i-n-g-e-r. I am here to talk about Pompe disease. My daughter Parker, back there, she's two. We welcomed her into the world January 31, 2015. She was eight and a half pounds, so she was a big baby, had a lot of energy stores in there. At first she seemed normal, healthy. She passed all of her normal newborn tests with flying colors. But within a couple months, she began to nurse not quite as well. She would only nurse for two to three minutes at a time and almost always fall asleep after that first two to three minutes. I mentioned it to her doctor, that she'd been sweating and nursing not as well. And of course, she couldn't see it right then, so she said: just keep an eye on her. I am a nurse, so she knew that Parker had somebody that knew what to look for. So then, after that, Parker began vomiting large amounts after every feed, so she sent us to a pediatrician. And the pediatrician...we mentioned the symptoms to him. And she'd just finished feeding, so he could actually see that, as well. And he did an electrocardiogram and an x-ray. And on the x-ray, at three months, her heart was the size of a ten-year-old's heart. So obviously something was wrong. He set us up with Dr. Fletcher from Children's, who was going to be in Kearney the following week. We went and saw Dr. Fletcher. He did an echocardiogram...stayed in the room

for about a minute, and then he left, said he'd see us when he got us back to the room after the test. Following the test, we went in there and we found out that he'd left the room early to go arrange transport to Children's because he didn't know why her heart was that big, but that she might only have a couple days if we didn't act quickly. We got to Omaha and, at first, Dr. Rizzo had mentioned to the other cardiologist that he suspected Pompe disease. But she didn't have the facial symptoms of the other patients they had seen with Pompe disease. So they thought that it could be something else. Then her initial testing at Children's had come back as a false negative. So after that, we had her go up for a test the following day, and that morning was the last morning, for two weeks, that we would see her breathing on her own without any assistance whatsoever. When she finished her surgery for more testing that morning, she came back on a breathing tube, and they were unable to get her successfully off for a little while. On May 13, 2015, Parker coded, meaning her heart went into a negative rhythm and her heart then stopped. And they worked on her for four minutes before they were able to get her back. That was a very long night for all of us. Thankfully my husband and my mom were both there, and I wasn't at Children's alone with her. The following day they gave us great news. They'd sent off some muscle specimens, and she did, in fact, have Pompe disease. Two independent labs had...sorry. Two independent labs had diagnosed her Pompe disease, so we knew for sure that that's what was causing all of her symptoms. After that we learned a lot of different terminology. And Dr. Rizzo came to rush her enzyme treatment so that we could get it in emergent situation, because it is very expensive. But without it, we honestly didn't know how much longer we would have after that, as well. Then in June, she got a tracheostomy placed because her heart was so big, it was collapsing her left lung. Following the tracheostomy, she got a port placed because this is an IV medication that she has to get, and the port allowed them to put the medicine in through there. In August, her bowel had not had enough blood flow, due to the disease and the advancement of her heart disease. So her bowel ruptured, and we had to go in for another surgery...got a feeding tube because she still wasn't able to eat. Since then she's been doing great. We were able to come out of the hospital after five months of hospitalization in Omaha, about five hours from home. She pulled her own trach out in March of last year, so she no longer has that. And she's breathing just fine, just has oxygen at night. But had we known, at birth or shortly after birth, that she had Pompe disease, we could have avoided the trach, the feeding tube. She wouldn't have coded that night. She would still have Pompe disease, but we would have a much better understanding, from the get-go, and get her help as soon as possible. Our child also has Pompe disease, and it will be nice to see what the difference is between the two, being able to start from the beginning. So thank you. Do you have any questions? [LB91 LB401]

SENATOR RIEPE: Thank you very much for your testimony. I apologize for having to step out, but I got called out, so... [LB91 LB401]

ASHLEE SPRINGER: That's okay; thank you. [LB91 LB401]

SENATOR RIEPE: Are there questions from the committee? Thank you for telling your story. [LB91 LB401]

ASHLEE SPRINGER: Okay. [LB91 LB401]

SENATOR RIEPE: Additional proponents. Proponents? Seeing none, do we have opponents? Any opponents that will be...want to speak? Seeing none, are there any that want to testify in a neutral capacity? Apparently not. Tyler, do we have any written documents that have been submitted? [LB91 LB401]

TYLER MAHOOD: (Exhibits 3-10) Yes, for LB91: we have a letter submitted, in support, by Dr. James Harper, representing himself; and Matt Keppler, a letter of support from the March of Dimes. For LB401: a letter from...a letter of support from doctor--not doctor--from Robert Rauner of the United Leukodystrophy Foundation; a letter of support from Dr. Richard Azizkhan from Children's Hospital; a letter of support from Chelsea Lamers, representing herself; a letter of support from Darin Anderson, representing himself; a letter of support from Matt Keppler of the March of Dimes; and a letter from Daniel Claussen, representing Nebraska Medicine, in support. [LB91 LB401]

SENATOR RIEPE: Okay. Thank you, Tyler. Senator Hilkemann, would you like to close? [LB91 LB401]

SENATOR HILKEMANN: Thank you. Well, you've heard the stories. You've heard the need. Senator Williams, the...if you look at the fiscal note, that $20, it gets us to $270,000. And that's where we can have some discussion on that down the line (inaudible). All of these parents who came here today with their children are here, not because their children can be cured. They came here to testify. They want to help other families prevent so that they don't have to live the challenges that they are living with in their lives. I think we need to make this a priority and move it forward. With that, I would close my testimony. [LB91 LB401]

SENATOR RIEPE: Thank you. We'll see if there are...Tyler, what do you have? [LB91 LB401]

TYLER MAHOOD: Can I correct the record real quick? The letter from Nebraska Medicine was in the neutral, not a letter of support. I apologize for that. [LB91 LB401]

SENATOR RIEPE: Okay, thank you. Are there additional questions of Senator Hilkemann, from the committee? Senator Crawford. [LB91 LB401]

SENATOR CRAWFORD: Thank you. And thank you, Senator Hilkemann. The letter that we did just get from Nebraska Medicine and just had mentioned the Medicaid program reimbursement and if you...and the possible need to consider adjusting that to reflect the cost of the tests, are you interested or willing in having that conversation? [LB91 LB401]

SENATOR HILKEMANN: We will have that conversation. [LB91 LB401]

SENATOR CRAWFORD: Excellent. [LB91 LB401]

SENATOR HILKEMANN: That's why they have...I am on Appropriations here, Senator. [LB91 LB401]

SENATOR CRAWFORD: Thank you. [LB91 LB401]

SENATOR RIEPE: Okay. Additional questions? Hearing none, that will close the hearing on the combined LB91 and LB401. Thank you, Senator Hilkemann. We will now hear, go into hearing on LB481, and this is Senator Kuehn. Do I see... [LB91 LB401]

SENATOR WILLIAMS: Want to take a quick break?

SENATOR RIEPE: Let's take a quick break. Can you see if we can get a hold of Senator Kuehn and tell him we're ready?

BREAK

SENATOR ERDMAN: Okay, we'll get started. Senator Riepe had to step out for another committee hearing. So Senator Kuehn, would you like to introduce LB481? [LB481]

SENATOR KUEHN: (Exhibit 1) Thank you, Senator Erdman and members of the Health and Human Services Committee. My name is Senator John Kuehn, J-o-h-n K-u-e-h-n, and I represent Legislative District 38, which is 7 counties in south central Nebraska. It's my pleasure to be with you this afternoon, to introduce LB481. I think it's highly appropriate that we're doing LB481 today on Groundhog Day. As some of you who are returning to the committee may remember this bill as LB979 from last year, which was passed out of this committee and advanced to General File, but was not taken up on the floor when we adjourned sine die. So it's very appropriate that, on Groundhog Day, we're literally having a Groundhog Day experience. LB481 is a bill dealing with biologics, which are an innovative class of medicines manufactured

from living organisms. Unlike small molecule drugs that are chemically synthesized, biologics are derived from cell lines that are ultimately going to produce the desired therapeutic substance. While most of the drugs that you are going to commonly think of are chemically identical batches, the biologics that we are going to be talking about today with LB481 are complex, heterogeneous mixtures. While they may have the same treatment and therapeutic uses, biologics produced using different cell lines will not be exactly the same. What we're going to be dealing with in LB481, and you'll be hearing about today, are biosimilars, which are also referred to as follow-on biologics. These are biologicals that are manufactured using different cell lines, mirroring the composition and treatment profile of an innovator product produced by another company. Biosimilars present therapeutic and cost-effective alternatives to innovator products for both providers and patients. Now I am going to ask if you indulge my inner geek here a little bit as we walk through a little bit about what biologics and biosimilars are. So while we're going to be talking about some regulatory aspects, it's important that you guys have some background on exactly what we're talking about with this new innovative class of therapeutics. Biosimilars are a relatively new development in therapeutics. And only recently has FDA guidance on their interchangeability been defined, with updates coming as recently as the past few weeks. LB481 is important as a piece of legislation for Nebraska, to help provide guidance for clinicians and dispensers, as we see more and more biosimilars pass through the FDA approval process. And we now have a mechanism in place for the concept of interchangeability. It's important that transparent communication between the patient, the physician, and the pharmacist remain the hallmark of high-quality patient care. What LB481 does is provides guidance to facilitate the communication when an approved interchangeable biosimilar product is dispensed by, and substituted by, the dispenser. While currently we don't have any approved interchangeable biosimilar products, there are many in the pipeline. And so what we're doing here is we're proactively, as a state, establishing a communication framework for the use of interchangeable biologics so that we have a commonsense set of solutions for promoting patient understanding of their own healthcare and communicating between all members of the healthcare team, so the prescriber, the patient, and the dispenser. I have for you, which your page can hand out, a table which includes all of the states that have adopted similar legislation. So while the FDA is ultimately the federal agency responsible for the approval of biologics and biosimilars, and approving the interchangeability, it's up to state law to govern the substitution by dispensers when a different product, other than that prescribed, is substituted, which is why we're here today. It's really important that we understand the role that biologics are going to play in treatments, and why this is such an important issue, especially the communication piece. The biologics that we're going to be talking about--and the experts will be providing testimony on that follow--are used in some of the most complicated medical conditions. So these are patients with really complex cases who are on multiple drug therapies and for whom management of their complex cases is critical. We're talking about cancer patients; we're talking, in many cases, about complex autoimmune diseases such as lupus and rheumatoid arthritis. And these are patients who are really taking advantage of these innovative therapies which, in case of

management and treatment of their condition, has been life changing. In this particular patient population, adverse event reporting and having a complete and accurate medical record is essential to the management of their conditions. These are also patients for whom their treatment programs are some of the most costly, in terms of both drug therapies and duration of their conditions. So making sure that we have a framework in place to allow lower cost alternatives for critical and affordable care is important. The second thing I want to make sure the committee is familiar with is that biosimilars is not the same as generic drugs. So generic drugs are chemically identical. By the very nature that they're produced by living cell lines, biosimilars are not. So while biosimilars and the interchangeables are approved for treatment of the same condition, and they're expected to have the same therapeutic use, they're not chemically identical. So it's for that reason, and because of the targeted population in which these compounds are used, that the healthcare team must clearly communicate the specific biologic and the manufacturer to both the patient and the physician, to maintain quality case management. So to achieve these objectives of having the best possible communication among members of the healthcare team and to advocate for the patient, as well as provide guidance for interchangeability, LB481, as the green copy in front of you contains the following provisions: First, it establishes definitions for biologic and biologic product. Given that these are a new and unique therapeutic class, definitions provided in state statute are important. And I'd like to give a strong thank you to all of the stakeholders, whether that is the pharmacists, the pharma companies, all of those advocating in this world of therapeutics, touching every aspect of biologics for coming together to establish and define these terms for us in state statute. Second, LB481 delineates clear guidelines for communication between the dispenser and the prescriber, in the event of the substitution of an FDA-approved, interchangeable biosimilar. So the language of the bill would require communication within three business days of a substitution by the dispenser, and it outlines the means for electronic communication of that notification piece. Third, LB481 is going to instruct the Department of Health and Human Services to maintain a list of the FDA-approved, interchangeable biologics, so that all members of the healthcare team have equal access, via the Web site, to identify specifically which products have received that interchangeable designation. And finally, LB481 does an important distinction in bringing Nebraska state statutes up to date regarding the differences between a drug and a biologic. Because biologics, including biosimilars and interchangeable products, are not identical to the reference products, by their very nature, and don't have the same active ingredients, like generic drugs, we need to provide those definitions. Current state law only allows a pharmacist to substitute a drug that is chemically equivalent and bioequivalent. So we have that important change to adjust for the change in technology. Following my opening are a number of nationally recognized experts who can provide additional technical expertise in the use of biologics, and their substitution, to the committee. While I recognize the nature of this discussion can be quite technical, I am happy to answer any questions that you may have, to the best of my ability, or to find out additional answers from the technical experts that follow. So with that, I am open to any questions you may have. [LB481]

SENATOR ERDMAN: Any questions? Senator Crawford. [LB481]

SENATOR CRAWFORD: Thank you, Senator Erdman. And thank you, Senator Kuehn. The text of the bill also has definitions for brand name and... [LB481]

SENATOR KUEHN: Um-hum. [LB481]

SENATOR CRAWFORD: ...and chemically equivalent and generic, which seem to be different than this biologic/biosimilar conversation that we're having. Is that updating that language for the Pharmacy Act (sic: Pharmacy Practice Act) more broadly, or is that something that's in there, in this bill, for another reason? [LB481]

SENATOR KUEHN: Yeah. No, it's updating the language to just reflect the role that we now have to update the entire pharmacy component with chemical equivalents and generics was along with biologics. So it's just standardizing definitions so that all individuals, dispensers, as well as prescribers, are clear on the distinct difference between these two classes of drugs. [LB481]

SENATOR CRAWFORD: Great, thanks. [LB481]

SENATOR KUEHN: All right. [LB481]

SENATOR ERDMAN: Senator Howard. [LB481]

SENATOR HOWARD: Thank you, Senator Erdman. Thank you, Senator Kuehn, for bringing this bill again. We had worked on it last year. Last year I remember there was an issue with the pharmacists. [LB481]

SENATOR KUEHN: Um-hum. [LB481]

SENATOR HOWARD: And that revolved, I think, a little bit about...around that notice piece. [LB481]

SENATOR KUEHN: Um-hum. [LB481]

SENATOR HOWARD: And so can you talk about, to the best of your knowledge, how that might work, in practical terms, for our pharmacists? [LB481]

SENATOR KUEHN: Yeah. So I believe you will have a letter of support that was submitted, to legal counsel from the pharmacists, which I...I am really grateful for their willingness to work and understand that notification piece. In practice, this notification is going to happen via the electronic system. So the PDMP and other great systems that we have in front of us and available to us are going to be key in that electronic notification piece. So the information of the substitution will be entered into the electronic system, and the prescriber has the ability to identify if that change is made. I think it's also important so that everyone is clear, the physician included, whether it's a generic drug or a biologic interchangeable, still has the full ability to do a DAW, or dispense as written, meaning that a substitution would not be allowed. So while this is not an active communication piece, it would be what we would consider to be a passive communication piece; it's in the electronic medical record. If the prescriber has significant concerns, they still remain fully in control of ensuring that the prescribed-as-written product is the one that is dispensed by the dispenser. [LB481]

SENATOR HOWARD: Thank you. [LB481]

SENATOR KUEHN: Um-hum. [LB481]

SENATOR ERDMAN: Any other questions? I just have a...I guess I have a comment, Senator Kuehn. I see on the information you've given us that, as time has passed from 2013, '14, and '15, the passage rate, or those who vote against passing this, seems to be narrowing (laughter). The last six or seven have been all zeroes. So they're telling the story better to some people, I believe, and they're understanding it. [LB481]

SENATOR KUEHN: Yeah, I think...I think also it's just some of those early challenges were just trying to explain what this class was, because it's important to recognize this as an innovative area of therapeutics. And so when you're talking about products to which are only a handful on the market, it was difficult to explain the need for this type of legislation. I think you're going to hear today from some individuals who have benefited from these products. And it's been a game changer in terms of the management of their medical condition. And that certainly has created a greater sense of urgency, as well as an understanding of the need for this legislation at the state level. [LB481]

SENATOR ERDMAN: Thank you for bringing it. Any other questions? Thank you very much. [LB481]

SENATOR KUEHN: All right, thank you. [LB481]

SENATOR ERDMAN: Any other proponents? Thank you for coming. [LB481]

DR. BRAD JORDAN: Good afternoon. [LB481]

SENATOR ERDMAN: Good afternoon. [LB481]

DR. BRAD JORDAN: Thank you for having me today. Mr. Chairman, members of the committee, my name is Brad Jordan. I am a scientist with Amgen. We are a United States-based pharmaceutical company, and we manufacture innovative biologic therapies, as well as biosimilars, which we're here to discuss today. So I... [LB481]

SENATOR ERDMAN: Could you...could you spell your name for us, please? [LB481]

DR. BRAD JORDAN: Oh, I am sorry, yes. B-r-a-d J-o-r-d-a-n. [LB481]

SENATOR ERDMAN: Thank you. [LB481]

DR. BRAD JORDAN: Sure. So I am here mainly as a scientist today. So I am...I will be happy to answer any scientific questions you have about biologics or biosimilars. But I also want to emphasize, you know, why we are here today, so that the passage of this legislation is an important update to some rules that are already in existence covering generic substitution. So in 2010 legislation was passed that allowed biosimilars to be accommodated in the U.S. marketplace. And so typically what that means is that a pharmaceutical company can make a...sort of a copy of what is already on the market. Okay, so after it's been on the market for a period of time, a company can make its own version of that molecule. So many companies can do this at one time. So there might be many biosimilars to one reference product, which is what we call the original innovator product. So something may be a biosimilar, but the FDA can also make a determination that that biosimilar is an interchangeable biosimilar. And there is additional evidence required to do that that was laid out by FDA in some draft guidance recently. But what that means is that, if it's designated as interchangeable, that the prescription that the physician writes can be substituted, at the pharmacy level, by a pharmacist. So if they write for the brand name, then the interchangeable biologic could be substituted. Okay? So the reason we're here is because the way these molecules behave is distinctly different than a generic molecule...than a generic brand name chemical entity. So you already heard from Senator Kuehn that chemical drugs are synthesized through discreet chemical reactions, and they can be made reliably, identical every single time. Okay, biologics are synthesized by living cells, and they're not identical every time. So one manufacturer's version is going to be slightly different than another manufacturer's version. But they're still deemed safe and effective by FDA. They're

rigorously reviewed for safety and efficacy, and so they're expected to perform in a very similar manner. But when you have an interchangeable, this is where things can be substituted at the pharmacy. And so we need laws in place that basically enhance the access of patients to these biosimilars, because they're potentially lower cost; they will save patients money. But also we want to make sure that the communication lines are maintained between the prescriber and the pharmacist, as well as the patient, to keep everybody involved in the care because, as you've heard, these are typically grievous illnesses, and it's typically not just to give you a pill and you go home and you're fine. So I want to point out some differences between the molecules and why we need these laws to support this. So biologics...if you think about a small-molecule drug--let's just say aspirin, for example--so if you maybe picture an aspirin molecule as the size of a golf ball, okay...a biologic might be the size of an F-150. So it's huge compared to a small molecule, and so in your body, the small molecule may go completely unnoticed. It does its job, it works, you never...your body never reacts to it. I mean the therapeutic benefit is there, but your body doesn't respond. Now with a biologic, it can be recognized by your immune system and so, therefore, you may have...a patient may have an adverse reaction to this biologic. And since there's going to be multiple versions of the biologic on the market, it's important to be able to...for the physician to be able to notice exactly what...to know exactly what brand the patient received. So this legislation that we're passing today, or that we're proposing today, is mainly to support patient access to these biologics to maintain lines of communication between physicians and healthcare...the healthcare providers and the pharmacists. We want to make sure patients know that they're getting a substitute, a biologic, and we want to make sure that there are accurate medical records that are kept so this can be traced back to the original manufacturer. So this is a very important piece of legislation that supports patient access, it promotes safe and appropriate use, and it will allow for accurate monitoring to ensure accountability. Now this legislation that you're seeing is developed nationwide through a large coalition of manufacturers, patients, and pharmacists, and doctors. It's already been passed in 27 states, in addition to Puerto Rico, and it's, you know, it's really just updating the laws to cover biologics from the original generic laws. Okay? And I am happy to answer any questions you have, scientific or about F-150s (laughter). [LB481]

SENATOR CRAWFORD: Hmm. [LB481]

SENATOR ERDMAN: Senator Kolterman: [LB481]

SENATOR KOLTERMAN: Thank you, Senator Erdman. Thank you for coming today... [LB481]

DR. BRAD JORDAN: Sure. [LB481]

SENATOR KOLTERMAN: ...to testify. Does...how does the insurance company look at this versus...name brand versus generic, from the perspective of...is there a lot of potential to save dollars? [LB481]

DR. BRAD JORDAN: So there is the potential savings. So there are four approved biosimilars in the United States. The savings are really undetermined yet, because there's really not...all of them are not on the market. So we don't know exactly what the savings could be, but there are potentially big savings. [LB481]

SENATOR KOLTERMAN: Okay. Thank you. [LB481]

DR. BRAD JORDAN: Yes. [LB481]

SENATOR ERDMAN: Senator Williams. [LB481]

SENATOR WILLIAMS: Thank you, Senator Erdman. And thank you for being with us. [LB481]

DR. BRAD JORDAN: Sure. [LB481]

SENATOR WILLIAMS: You mentioned, as I understand the science of this, that the biosimilar will not be exactly the same chemical like we talked about with the generics. [LB481]

DR. BRAD JORDAN: That's right. [LB481]

SENATOR WILLIAMS: But if your company, Amgen, is producing a biosimilar, will your biosimilar always be the same, as when you produce it on Monday, as it is on Wednesday? [LB481]

DR. BRAD JORDAN: So that's a good question. So inherent to the production of a protein therapeutic, which is what a biologic is, there is inherent variability because the cells...basically the protein is made by cells, it's harvested away, and it's actually very dependent on the environment that the cells are cultured in, as well as the manufacturing process. And so a company like Amgen, we have very rigorous controls in place throughout the manufacturing process. And so, batch to batch, these things are monitored very carefully. And so you are...you have to fit within a prescribed margin, according to FDA standards; it's actually an international standard that we use. But they're never identical, but they are very, very similar from the same

manufacturer. But they may be different from a different manufacturer, but they're still similar to the reference product. [LB481]

SENATOR WILLIAMS: They're all meeting the standard that the FDA has put there. [LB481]

DR. BRAD JORDAN: That's right. Absolutely, correct. [LB481]

SENATOR WILLIAMS: So there would be no issue with that. [LB481]

DR. BRAD JORDAN: No, not at all. [LB481]

SENATOR WILLIAMS: Thank you. [LB481]

DR. BRAD JORDAN: No, they're rigorously tested. [LB481]

SENATOR CRAWFORD: That's okay. [LB481]

SENATOR RIEPE: Go ahead. [LB481]

SENATOR ERDMAN: Thank you. So as some of these have been in place for three or four years, so the results that you've seen from those applications in those states that were approved several years ago, are those results known to people? [LB481]

DR. BRAD JORDAN: Well, there's no...are you talking about the therapeutic results or the interchangeables? [LB481]

SENATOR ERDMAN: Yeah. Therapeutic, yeah. [LB481]

DR. BRAD JORDAN: So we don't have any interchangeable biologics yet, so there's no interchangeable biosimilars that have been... [LB481]

SENATOR ERDMAN: Okay. [LB481]

DR. BRAD JORDAN: ...designated by the FDA. But biosimilars have been using Europe for going on ten years now. So it's, you know, it is a...it is a viable market, and it's, you know, really it's a fledgling market in the United States, but it does have the potential. And these molecules...I

am an analytical scientist. They are very rigorously tested by the FDA, or assessed by the FDA for their safety and efficacy. [LB481]

SENATOR ERDMAN: All right. Thank you. [LB481]

DR. BRAD JORDAN: Sure. [LB481]

SENATOR RIEPE: Senator Crawford. [LB481]

SENATOR CRAWFORD: Thank you, Senator. Thank you; thank you. So just to clarify, we have biosimilars. [LB481]

DR. BRAD JORDAN: That's right. [LB481]

SENATOR CRAWFORD: But they have...but the interchangeable is a higher standard that you have to get from the FDA. [LB481]

DR. BRAD JORDAN: You have to provide additional evidence. [LB481]

SENATOR CRAWFORD: Right. [LB481]

DR. BRAD JORDAN: And the definition for an interchangeable is that it has to produce the same result in any given patient. And so there are...is additional evidence that has to be submitted to FDA to support an interchangeability designation. Now that guidance is not finalized; the draft guidance for interchangeability was just released by FDA about a week and a half ago. So there's a period of time where scientists and companies will comment on that, on the guidance, and it may be amended to include additional things, you know. But it's actually very rigorous to be assessed as an interchangeable. [LB481]

SENATOR CRAWFORD: So the reason we don't have interchangeable products yet is that the regulations haven't been clarified yet, not that we haven't created molecules that could pass that test. Is that correct? Or we don't know. [LB481]

DR. BRAD JORDAN: I don't think we know the answer to that. [LB481]

SENATOR CRAWFORD: Okay. [LB481]

DR. BRAD JORDAN: So I mean the evidence is very rigorous, but it's very likely that an interchangeable could be designated this year. [LB481]

SENATOR CRAWFORD: Okay. [LB481]

DR. BRAD JORDAN: I mean...so the important thing to remember is guidance by FDA is just guidance; it's not rules. [LB481]

SENATOR CRAWFORD: Hmm. [LB481]

DR. BRAD JORDAN: So it doesn't mean that you have to absolutely accomplish this to be declared an interchangeable. So you could see an interchangeable on the market this year--next month, whenever. You know? The guidance does not have to be final before they declare an interchangeable. [LB481]

SENATOR CRAWFORD: Thank you. [LB481]

DR. BRAD JORDAN: Um-hum. [LB481]

SENATOR RIEPE: Additional questions? Hearing none, thank you very much. [LB481]

DR. BRAD JORDAN: Sure. [LB481]

SENATOR RIEPE: Additional proponents? I will give you a heads up, too, with our speaker coming up--the young guy over here is going to take a photo. So if you see him walk up here behind Senator Erdman, then that's why. Okay, thank you, sir, for being here. Please introduce yourself and spell your name and go forward. [LB481]

DR. HARRY GEWANTER: Thank you. My name is Dr. Harry, H-a-r-r-y Gewanter, G-e-w-a-n-t-e-r. Mr. Chairman and committee members, thank you for allowing me to come today and speak on...in support of LB481. And this is legislation that's going to affect, really, thousands of Nebraska residents. I am a pediatrician and pediatric rheumatologist from Richmond, Virginia, with over three decades of experience of caring for children with rheumatic and other serious and chronic illnesses. I serve as the chairman of the Alliance for Safe Biologic Medicines, or ASBM. And ASBM is an organization of organizations of physicians, pharmacists, patients, manufacturers of both innovator products as well as biosimilars, researchers, and other organizations, that we're all working together to ensure the patient's safety is at the forefront of

all biosimilar discussions. As you've heard, biologic medicines are complex molecules that have really revolutionized the treatment of a lot of chronic and serious diseases, you know, such as cancer, rheumatoid arthritis, juvenile arthritis, Crohn's disease, psoriasis, and multiple sclerosis, to name a few. And I can personally tell you that I am old enough to have started treating people before we had anything that really worked on kids with arthritis. And I really consider these things miracle drugs. I look at my career as being, probably, equivalent to physicians in the '50s and '60s, when they had antibiotics and chemotherapy, and you had conditions that you had nothing you could do about. And now, all of a sudden, you could treat people. The walkers and wheelchairs that I would see at the Arthritis Foundation's juvenile arthritis meetings 20 years ago are essentially nonexistent today. And actually the biggest problem now is we've got kids running around the hallways and they've got to be told to stop (laughter). You know, this is really a nice problem. My field also has to sit around; we have to start thinking about what a week to find a cure, because it's potentially there. And again, this is another nice problem. And this is what biologic medicines have brought to our field. You've already heard about how biosimilars cannot be exactly the same, and Dr. Jordan has talked about things on a biologic and molecular level. I want to talk about it really, again, as a clinician. One of the things to realize...you know, we are using some very complex, very high-tech and, admittedly, very expensive medications to treat very serious and chronic illnesses that are disabling and, if not treated appropriately, will cause crippling or other problems. So for example, if you have multiple sclerosis and your disease is not controlled and you have a neuron that dies, it's not coming back. In my conditions, if your cartilage or your bone goes away, it's not coming back. And so the revolution from these medications is that we are now able to target specific processes in the body, shut them down or diminish them, and prevent the damage that comes from your immune system overdoing things. It's extraordinarily important that we have the tracking and notification and communication among the healthcare team, as was mentioned, because, in my field if someone is not doing as well, if they've been on a medicine for a while, I just assume they've stopped taking it. I mean, kids do that; former children do that, too. But I think part of the thing is I had to be able to know if the medicine was switched, I have to be able to factor that into my decision-making. about what's going on. And that's why I think the communication among the healthcare team is extraordinarily important. As you know, current Nebraska law does not have a specific pathway regulating the substitution of medicine with biosimilar, and LB481 will just update and bring the Nebraska's Pharmacy Practice Act into current times. Even though there's not something yet, it's going to be coming very soon. And I think that it's very important this is passed and, you know, I encourage you to do so and we fully support it. There are a few things in the bill that are extraordinarily important, from our perspective, as well. Again, it provides that only interchangeable biosimilars may be substituted. It allows the physician, again, to specifically define which medication he wants, patient's (inaudible) physician and the patient together have decided on what treatment they think is most appropriate. It allows the patient to also choose and has the option to prevent a substitution. And again, most importantly, within a reasonable time, three business days, the pharmacist can communicate to the physician which medicine was

actually dispensed. And records will be kept so that, if there are problems or, you know...the other thing is every single patient is a unique chemical experiment. There may be certain biosimilars that, in fact, work better for a certain group of people than for others. And this would allow us to learn from that and to improve our treatments for these significant and serious illnesses. We've done many studies throughout both the United States and the world, and the physicians are overwhelmingly in favor of having this communication between all members of the healthcare team. And this really helps to promote the physician/patient relationship. So again, support LB481 and, because it will extend these valuable protections to Nebraska citizens, and while increasing their access to these biologic medicines. Again, 27 states and Puerto Rico have passed very similar legislation, and this is really a preventive act on your part, too, to be ready for when these medications are there. [LB481]

SENATOR RIEPE: Okay. [LB481]

DR. HARRY GEWANTER: So I thank you very much and I'll answer any questions. If you'd like, I can address some of the questions that were asked earlier about the savings and the interchangeabilities, if you'd like. [LB481]

SENATOR RIEPE: We thank you for being here. We're going to open it up now for questions. [LB481]

DR. HARRY GEWANTER: Okay. [LB481]

SENATOR RIEPE: And I see one with Senator Williams, over here, to start with. [LB481]

DR. HARRY GEWANTER: Sure. [LB481]

SENATOR WILLIAMS: Thank you, Chairman Riepe. And first of all, would you answer those questions (laughter)? [LB481]

DR. HARRY GEWANTER: Absolutely. I think part of the question with the savings is we are not going to see the same degree of savings that we see with generics and where prices went down 50 percent or more. Currently it's about 15 percent, and it looks like differences between originator charges and biosimilar charges. On the other hand, you know, 15 percent of $100,000 is real money, as well. I think we're going to start seeing more savings when there is more than one biosimilar on the market. So you now, instead of having two products competing, you're going to have more than two. But that's going to take a while. So that yes, there's going to be

significant savings. I don't think it's as much as advertised, at least yet. But it's definitely going to be there because these are so expensive to develop, produce, and use. [LB481]

SENATOR WILLIAMS: Okay. [LB481]

DR. HARRY GEWANTER: Does that help answer you? [LB481]

SENATOR WILLIAMS: Certainly it did. My real question goes to the...you're a clinician who would be prescribing these. [LB481]

DR. HARRY GEWANTER: Yes, sir. [LB481]

SENATOR WILLIAMS: And the system with the three-day period and also, as I understand it, you could put on your prescription that you don't want a substitute and that and that. [LB481]

DR. HARRY GEWANTER: Correct. [LB481]

SENATOR WILLIAMS: Can you just walk through those processes and how it really works? [LB481]

DR. HARRY GEWANTER: Well, we haven't had to do it much yet. But I think... [LB481]

SENATOR WILLIAMS: No, how you expect that to work. [LB481]

DR. HARRY GEWANTER: I am...there are a number of my colleagues that would like to be able to say, specifically: I want to give approval before this happens. I guess I am maybe a little more humble about my predictive abilities. Within three days, to me, is sufficient because a patient is not going to get more than one or two doses in those days. And for me to know that things have been changed allows me to then factor that into my observations and decision-making for that patient. So I really don't know. A biosimilar may be better for that specific patient than the originator. And that, you know, may work out, you know, better for everybody. But if there's...so I am more interested in the communication issue. I think there are certainly some situations where I may say, you know, for instance, a biosimilar may come only as a prefilled syringe, whereas the originator has a vial or an AutoSyringe. And for that particular family, they're going: we cannot administer this syringe, it just ain't going to happen; in which case I must say: okay, we need to do the vial or we need to do the autoinjector, which would be,

you know, the brand name product. So you know, I can foresee a number of situations like that where both the delivery system, as well as the specific product, may be an issue. [LB481]

SENATOR WILLIAMS: Wouldn't it also be possible that you have someone that you are treating and the--I'll use the term--brand name is working very well. [LB481]

DR. HARRY GEWANTER: Correct. [LB481]

SENATOR WILLIAMS: And you don't want to risk... [LB481]

DR. HARRY GEWANTER: Absolutely. [LB481]

SENATOR WILLIAMS: ...and you wouldn't have to, under this legislation. [LB481]

DR. HARRY GEWANTER: That is true. Now whether the payer would agree with all of this is a whole "nother" discussion, but I would have the ability to say: yes, I want to stay with this. I can give you an example from the last century, with chemical drugs, where there were some antibiotics that came out that were very effective for ear infections and all sorts of other things. And they're still being used significantly for urinary tract infections. And the brand names tasted delicious. There was a grape flavor, there was a strawberry flavor; it was wonderful. Kids had no problems taking it. Generics came out and didn't taste so good. And all of a sudden I...and I hadn't realized it. And I had parents coming to me and say: my child won't take the medicine; he spits it out...you know, he just won't do it. And it took a while to figure out what the issue was. And, literally, I had a pharmacist give me all their samples, and we taste tested them in my office. And I would then tell parents: you may want to pay extra for the brand because it's going to get down. I think there could be a parallel kind of situation here, as well. I would hope that most folks would be grandfathered in on whatever medicine, you know, by the pairs that they are currently on. I can see a number of physicians, as I've talked to them and we've done our surveys, who would not have a lot of problems about perhaps starting a new patient on a biosimilar. But I think a lot of us, for at least a few years, are probably have some hesitation about just automatically switching without some more data. [LB481]

SENATOR WILLIAMS: Thank you. [LB481]

SENATOR RIEPE: Okay. Thank you very much. Are there additional questions? Seeing none, thank you very much for being here with us. [LB481]

DR. HARRY GEWANTER: Thank you very much for the opportunity to speak to you. [LB481]

SENATOR RIEPE: Additional proponents? [LB481]

JACKIE NEWMAN: Thank you, Senator Riepe and the committee, for having me today. Excuse me. My name is Jackie Newman, J-a-c-k-i-e N-e-w-m-a-n. I'd like to begin by telling you about my daughter, Zoe. She was two at the time she was diagnosed with rheumatoid arthritis. She walked like a little old lady, and I couldn't put footie pajamas on her because she would cry because it hurt so bad. She also couldn't sit--at the time they called it crisscross applesauce--on the floor with her friends at daycare, nor could she get up because of her pain from the arthritis. So for the next two years, we tried the old drugs: the methotrexate, the Mobic, the ibuprofen, the naproxen. I am sure I've forgotten some of them. None of them worked for her. So finally, when she was four, she was old enough to try a biologic. And that biologic we call Zoe's miracle drug. So for the first time in her life, as a four-year-old, she was running. She was able to play with her friends and be a normal child. So this bill, LB481, is near and dear to my heart, of course, because it helps our daughter to function as her peers do. Fast forward to today, Zoe is now 12 years old, and she's playing basketball. And if you've seen seventh-grade basketball, it's much more physical than I remembered. She's playing soccer, beats on her little brother like any normal kid. Zoe also has a condition called uveitis, which is a type of arthritis in the eye. And if left undetected, untreated, she could lose her vision. Her biologic keeps that in check and makes it so that she doesn't have the flares and she doesn't lose her eyesight. So it has saved that for us. So as I said, again, it's very important for LB481 to pass. There is a significant--excuse me--a significant cost associated with biologics, but we feel very strong that Zoe is thriving because of the biologic that she's on, and thankful that we have health insurance that covers a large portion of that cost. Biosimilars and interchangeable biologics would lower these costs and make these treatments more available to more patients. A big concern of ours, regarding biosimilars and interchangeable biologics, is that communication piece between the providers, pharmacies, and the patient. These medications are very powerful, as we see every day, and often come with side effects and, as with any drug, have the potential of serious adverse effects. We are very fortunate and Zoe has not had any experience with a side effect because of her...from her biologic, as of yet. We've put our full trust into our physicians, and we always discuss Zoe's treatment plans at length before making a final plan. And what truly concerns us, in regard to the interchangeable biologics, is the chance that the dispensing pharmacy is not required to notify the physician or the patient when an interchangeable is used. If my daughter were to have a reaction, as her parent, I would need and want to know what medication she had been given. There'd be no way to track the side effects, in adverse events, to each particular biosimilar or interchangeable biologic if the patient and physician were unsure of what medication was actually administered. This would undermine the relationship between the physician and the patient. This is why I fully support the passing of LB481, with the communication component included. Thank you for your time. [LB481]

SENATOR RIEPE: Thank you. Are there questions? Seeing none, thank you for being with us. [LB481]

JACKIE NEWMAN: Thank you. [LB481]

SENATOR RIEPE: Additional proponents? [LB481]

ALLYSON JOHNSON: Thank you, Senators, for listening to me today. My name is Allyson Johnson, A-l-l-y-s-o-n J-o-h-n-s-o-n, and today I want to first start by talking to you about my disease. So it had taken 18 months for my family to find out what disease I had had. And at this time, I was very young. It was an 18-month stretch of my parents being told that I was faking my pain for attention, that they were the ones who were faking it for attention, and that there was just nothing wrong with me. Now this went on for 18 months, as I had mentioned twice before. And one night at my grandma's house, very late at night, I had had a pain-induced seizure due to my juvenile arthritis. So that led to my diagnosis at three years old. My doctor took one look at my hands and said to my parents: There's something wrong with your daughter. So in first grade, my nurse, my school nurse, had sat in my class with me and told everyone: Allyson is a little different; she has arthritis. And that was really embarrassing for me. So I wasn't a very large advocate until about seventh grade. Now in eighth grade I was almost in a medical remission, which is a very big deal for people with arthritis, and I had begun advocating, as I had mentioned, in seventh grade. But it got really big in eighth grade, despite all the people who told me: you don't really have arthritis; you're too young to have arthritis. And they just didn't believe me, and it was really hard to get that word out there. Now one story that I really like to think about sometimes is in track. I had just gotten my biologic shot the night before. And I was very competitive in middle school track, and I ran around that track better than I ever had before, after getting that biologic. So that was the most important day of track for me. Now in ninth grade, despite competing for the track season before that, I was told that I could no longer run. This was really, really devastating for me when it came down to the fact that I had competed for an entire year before that. And I was going into remission; however, my training that summer was so intensive that my arthritis had come back with a vengeance. It got so bad that, changing schools, I couldn't walk up the stairs at the high school without someone helping me or without holding on to that railing, because my knees wouldn't move, my ankles wouldn't move. But I found solace in advocacy. I started junior counseling at Camp Spirit, and this was the point where my advocacy became something bigger, something bigger than myself. It was for all of the children with arthritis because, if you've ever seen all of those kids at Camp Spirit, there's so much love and so much appreciation for one another, because we all know who is going through what. Now most people at Camp Spirit are able to walk normally. They're able to run and play. However, there are some girls who end up in wheelchairs, who can't run and play with all the other girls at the camp, who can't do all the other activities with the other girls at the camp. And this is because they aren't able to get the same medications that many of us are able to get. Now when

we consider the patient's...the patient's pharmacist and the doctor, we need to look at a story. About a year ago, I was actually really sick and I needed an antibiotic. And if it weren't for my patient (sic) taking a look at the different ingredients that was in that antibiotic, I actually could have died because of the reaction that it would've had with another one of the medications that I was taking. So that's something that we really need to keep in mind when we are talking about the communication, is because the biosimilars can have the same effect because of the way that they're made up. We need to make sure that we know how this is going to react with the patient's other medications and the way that the patient's disease works. So with that in mind, with hearing a personal story and hearing some of the things that could happen if we were to not pass this bill, I hope that you are in support of this bill, as well. [LB481]

SENATOR RIEPE: Thank you. You did a very nice job. [LB481]

ALLYSON JOHNSON: Thank you. [LB481]

SENATOR RIEPE: Are there questions? Senator Howard. [LB481]

SENATOR HOWARD: Thank you, Senator Riepe. Did you visit with us last year about this bill? [LB481]

ALLYSON JOHNSON: Yes, yes I did. [LB481]

SENATOR HOWARD: Okay. So what year are you in school now? [LB481]

ALLYSON JOHNSON: I am actually a senior. And this year I was actually able to run, and I did get fifth place at a meet for cross country. So... [LB481]

SENATOR HOWARD: Oh my goodness; that's wonderful. Well, thank you for visiting with us again. We appreciate it. [LB481]

ALLYSON JOHNSON: Yes, of course. [LB481]

SENATOR RIEPE: I have a question before you run off. [LB481]

ALLYSON JOHNSON: Yes. [LB481]

SENATOR RIEPE: And that was...I believe you said that some of the others...I think you said some of the other girls were not able to get the medication that you were able to get. Was that because they didn't qualify medically or for... [LB481]

ALLYSON JOHNSON: I am afraid I don't know the details for those other girls. However, I do know that they weren't able to find those medications. We, in the Arthritis Foundation, like to call them medicine cocktails because sometimes we have to just figure out what works for our certain disease, how it works for our certain disease. So they just haven't found their medicine cocktail yet. [LB481]

SENATOR CRAWFORD: Hmm. [LB481]

SENATOR RIEPE: Okay. I just wondered if it was a financial denial or something like that. Are there other questions of this young lady? Hearing none, thank you very much. [LB481]

ALLYSON JOHNSON: Thank you. [LB481]

SENATOR RIEPE: Additional proponents? [LB481]

PHIL KOZERA: (Exhibit 2) Thank you. Chairman Riepe, members of the committee, thank you for your time. I will keep my comments brief. I drew the short straw, having to follow Allyson (laughter), and I was just going to give a little bit of the industry perspective. [LB481]

SENATOR RIEPE: Would you give us your name, please? [LB481]

PHIL KOZERA: Yeah. My name is Phil Kozera, last name is K-o-z-e-r-a, and I am the executive director of the Bio Nebraska Life Sciences Association. [LB481]

SENATOR RIEPE: Okay. [LB481]

PHIL KOZERA: And we have a statewide membership of over 70 organizations that are making innovative products across the spectrum, from human health to medical device, animal health, plant sciences, renewable fuels. And we believe that the biosimilars legislation is important legislation. You know, it's important to the industry, it's important to the medical community and, most importantly, to the patients, as we've heard today. And I think it's unique for us to have such a broad coalition to come together for, you know, one specific proposal. But interchangeable biosimilars will be approved by the FDA, and I think the question that we have today is: Will

Nebraska be ready to help patients and doctors by defining a pathway of substitution for biological products in a treatment regimen? As you've heard, large molecule biologics are the future of precision medicine. In America alone, there are more than 900 biologics in development for more than 100 diseases. And thankfully, what was really a terminal prognosis 30 years ago, many are now manageable, thanks to advancements in medical research. With the promise of better understanding of genetics advancements, and coordinating wellness, and personalized medicine, we can create an environment in Nebraska where patients will have access to high-quality interchangeable biologic medicine. If you have questions about biologics, I put together a handout that explains the difference. We've had talk about large molecules, small molecule...and I have a reference document for you. As Dr. Jordan and Senator Kuehn mentioned, biologics are manufactured from living organisms, which is a big differentiator. They're hard to make and, as we've heard, they're hard to take. Each new therapy will likely cost nearly $2 billion, take over a decade to develop and get approved. And during this time, over 90 percent will fail. Due to the nature of biologics, we're asking Nebraska to outline the parameters for the substitution of interchangeable biologics to ensure that patients have access to high-quality, safe, and effective medicine. We believe the proposed substitution legislation , as we've heard from 27 other states, will enable Nebraska pharmacists to dispense effective and potentially less expensive biologic medications to patients, providing them with access to lower-cost treatments while really protecting the primacy of the physician and patient relationship. Again, thank you for your time today. Just in summary, you know, biologics are here, they're improving quality of life, extending survival and, in some cases, even curing serious disease. So thank you for your consideration of LB481. [LB481]

SENATOR RIEPE: Thank you for being here. We'll see if we have some questions. Any questions? I have a question. You had said, and I think you said FDA... [LB481]

PHIL KOZERA: Right. [LB481]

SENATOR RIEPE: I think the word that I heard was "will" approve, which means they haven't approved. [LB481]

PHIL KOZERA: They're... [LB481]

SENATOR RIEPE: So is this like a...and you talked about the very huge cost. And we all know how fast the FDA moves. Is this like a...shooting a clay pigeon, that you're...it's popping out there and we're trying to connect our state laws with when the FDA does approve? [LB481]

PHIL KOZERA: What I think is...Dr. Jordan mentioned, you know, there are several interchangeables in the pipeline and so, whether that's next month or next year, I think we'll see

these products here, simply because of the impact that the biologics have had. So this is, you know, in preparation of that opportunity. [LB481]

SENATOR RIEPE: I know there is some chatter, talk at the national level, about expediting FDA outcomes. Is that your experience, that you're seeing these things come out a little bit faster? [LB481]

PHIL KOZERA: Well, it's certainly our hope, simply because of the impact of the biosimilars, the impact of the large molecules on precision medicine. It's truly been a game changer. [LB481]

SENATOR RIEPE: Okay. Any other questions? Hearing none, thank you very much for being with us. [LB481]

PHIL KOZERA: Thank you. [LB481]

SENATOR RIEPE: More proponents. [LB481]

COLEEN NIELSEN: Good afternoon. Chairman Riepe, members of the Health and Human Services Committee, my name is Coleen Nielsen, spelled C-o-l-e-e-n N-i-e-l-s-e-n, and I am the registered lobbyist for Express Scripts. I don't have any substantive testimony this afternoon, but I am here to register our support for this bill. [LB481]

SENATOR RIEPE: Okay, thank you very much. Any questions? Hearing none, thank you very much for testifying. Additional proponents? [LB481]

BEREND KOOPS: Mr. Chairman, members of the committee, my name is Berend Koops, B-e-r-e-n-d K-o-o-p-s, and I am head of...or director, Merck government affairs for Nebraska. The same comments; I don't have any substantive; we just wanted to lend our support to the bill. [LB481]

SENATOR RIEPE: Okay, thank you. Any questions? Hearing none, thank you. Additional proponents. [LB481]

KATHY SIEFKEN: Chairman Riepe and members of the committee, my name is Kathy Siefken, K-a-t-h-y S-i-e-f-k-e-n, representing the Nebraska Grocery Industry Association. Many of our stores do have pharmacies, and we are here in support of the bill, would like to go on record as being such. If you have any questions, I'd be happy to answer. [LB481]

SENATOR RIEPE: Are there any questions? Hearing none, thank you very much. [LB481]

KATHY SIEFKEN: Thank you. [LB481]

SUSAN ZALENSKI: Mr. Chairman, I am Susan Zalenski, S-u-s-a-n Z-a-l-e-n-s-k-i. I am the director of state government affairs for Johnson and Johnson in the state of Nebraska. And we would like to go on...go on support of this bill, as well. [LB481]

SENATOR RIEPE: Okay, thank you very much. [LB481]

SUSAN ZALENSKI: Thank you. [LB481]

SENATOR RIEPE: We're moving right along here. Are there other...any questions from the committee? Hearing none, I...additional proponents? Not the last call, but maybe so. Any proponents? No. Hearing none, are there any speaking in opposition? Seeing none, are there any speaking in a position of being neutral? Seeing none, Tyler, do we have letters that have been sent in? [LB481]

TYLER MAHOOD: (Exhibits 3-15) Yes. The following letters are all in support: Michele Guadalupe of the Arthritis Foundation; Dr. Adam Reinhardt, representing himself; Marcia Horn of the International Cancer Advocacy Network; Nick Faustman of the American Cancer Society Cancer Action Network; Michael Stevens of the Coalition of State Rheumatology Organizations; Dr. Elena Rios of the National Hispanic Medical Association; Mark Davis of the National Kidney Foundation; Shaina Smith of the U.S. Pain Foundation Inc.; Theresa Morrow of Women Against Prostate Cancer; Randy Beranek of the National Psoriasis Foundation; Joni Cover of the Nebraska Pharmacy (sic: Pharmacists) Association; Dr. Todd Pankratz of the Nebraska Medical Association; and a letter submitted by the Pharmaceutical Research and Manufacturers of America. That's it. [LB481]

SENATOR RIEPE: Thank you. Senator Kuehn, would you like to close, please? [LB481]

SENATOR KUEHN: Well, certainly since the last few testifiers were quick, I have a lot of things I'd like to talk to you about (laughter), if that is...got some time if we're all a little early? [LB481]

SENATOR RIEPE: We have all day. [LB481]

SENATOR KUEHN: All right, no. Thank you. I appreciate your attention and your willingness to take up this matter today. I am happy to answer any questions that you may have. [LB481]

SENATOR RIEPE: Are there any questions? Evidently you did a good job. So thank you very much. [LB481]

SENATOR KUEHN: Thank you very much. [LB481]

SENATOR RIEPE: Thank you. That concludes the hearing on LB481. Thank you all for coming. We're now going to go into Executive Session. [LB481]