October 26, 2018

Dr. Kristina Box
State Health Commissioner
Indiana State Department of Health
2 North Meridian Street
Indianapolis, IN 46204

Amy Kent
Director of Legislative Affairs
Indiana State Department of Health
2 North Meridian Street
Indianapolis, IN 46204

Re: Support for Adding Pompe and MPS I to the Genomics and Newborn Screening Program

Dear Commissioner Box,

On behalf of the 1-in-10 Indiana residents with one of the approximately 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) writes in support of adding Mucopolysaccharidosis type I (MPS I) and Glycogen Storage Disease Type II (Pompe) to Indiana's Genomics and Newborn Screening Program (GNSP).

NORD is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. We are committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.

MPS I and Pompe are both rare disorders that if left untreated during the early stages of their progression can cause severe physical and developmental impairment in children as a result of cell and tissue degradation. Fortunately, improved treatment standards for each of these disorders, such as enzyme-replacement therapies, are available. Combined with a strong body of evidence demonstrating the effectiveness and reliability of newborn screening for MPS I and Pompe, these treatments make it possible for states to ensure the rapid identification and treatment of all newborns with MPS I and Pompe.

To make this a reality, however, states must screen for these disorders. By adding these disorders to its newborn screening panel, Indiana would be implementing a critical public health intervention to protect the approximately 1-in-100,000 babies born with MPS I and the 1-in-40,000 babies born with Pompe.

NORD recognizes that the state department's ability to add conditions to its panel can often be dependent upon additional support for program expansion, such as funding for lab improvements (including funding for personnel and new tests) and the implementation of proper follow up procedures.

To that end, we are supportive of initiatives to generate new legislative appropriations that will better enable the state department to add Pompe and MPS I to its panel. Moreover, we are interested in hearing directly from you or your program staff about how NORD can help address any other barriers preventing the department from adding these conditions. Please feel free to reach out to us directly to discuss this

further using the contact information below. We would be more than happy to meet with you to discuss the ways in which we can support your newborn screening program.

Thank you for your consideration of this letter and your ongoing support of the rare disease community in Indiana through the advancement of newborn screening.

Sincerely,

Tim Boyd, M.P.H., Director of State Policy
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Cc:

The Honorable Cindy Kirchhofer
Indiana House of Representatives
200 W. Washington Street
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The Honorable Ed Charbonneau
Indiana State Senate
200 W. Washington Street
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