



May 17, 2023

The Honorable Greg Gianforte
Governor, State of Montana
P.O. Box 200801
Helena, MT 59620-0801

Re: Serious Concerns Raised by SB 351 for Patients with Rare Diseases

Dear Governor Gianforte:

RAAP (Rare Access Action Project) just recently became aware of legislation which has the potential for devastating effects on people with rare diseases and respectfully urges you to veto SB 351, which risks curtailing Montanans' access to key clinical care and research opportunities. As detailed further below, the bill, as amended, could drastically limit genetic research that would lead to new therapies and cures benefiting individuals with rare diseases.

Patients with rare diseases face devastating obstacles. It takes them longer to be diagnosed, and, once diagnosed, they often have limited options for treatment. Of 7,000 identified rare or neglected diseases, only about 500 have approved treatments.¹

Genetic testing and research are vital to improve outcomes for these patients, as roughly 80% of rare diseases have a genetic basis.² Indeed, we have already seen that genetic research, and innovation in genetic research, holds the key for breakthroughs.

We respectfully urge you to veto SB 351 due to concerns about the impact of this bill on important genetic research for patients with rare diseases. The amendments to the bill seemingly broaden the scope of the bill to apply much more broadly than direct-to-consumer genetic testing companies to also apply to patient support groups, patient registries, and medical researchers who are performing life-saving genetic research to find treatments for rare diseases.

One limitation in rare disease research is the limited sample size of people living with the disease. To overcome this obstacle, patients and families are often eager to participate in patient registries and other opportunities to donate their genetic data and samples to future research. One example of such a registry is the DIPG/DMG Registry.³ This registry contains “comprehensive deidentified but linked clinical, imaging, .

¹ *Rare Diseases*, National Institutes of Health, <https://www.nih.gov/about-nih/what-we-do/nih-turning-discovery-into-health/rare-diseases> (last updated Feb. 28, 2023).

² *Rare Diseases Difficult to Diagnose, Cures Hard to Come By*, Associate of American Medical Colleges, <https://www.aamc.org/news/rare-diseases-difficult-diagnose-cures-hard-come> (Apr. 11, 2017).

³ DPG/DMG Registry, <https://www.dipgregistry.org/> (last visited May 16, 2023).

. . and genomic repositories” which it then makes available to researchers studying the disease.⁴ Patients, families, and researchers *want* this data to be accessed by as many researchers as possible, subject to appropriate safeguards, so that treatments can be found quickly.

However, SB 351 could be read to severely limit such research and bar Montanans from participating in registries and other research opportunities. The bill includes language that could be read to create a new, unworkable consent standard that involves every patient specifically consenting to each additional researcher who is provided access to their genetic data. This would foreclose patients – even those that provide their informed consent in accordance with human subject protection laws – from participating in these registries.

The bill also fails to distinguish between data that is linked to a patient’s name or other identifiers and data that has been de-identified pursuant to the Health Insurance Portability and Accountability Act (“HIPAA”) or other similar rigorous standards. This threatens to limit opportunities to conduct important research on de-identified data. Further, to the extent the bill could be read to require entities to honor revocation of consent or deletion requests for de-identified data, such requirements simply are not workable; it is not possible to link a request to their de-identified data in most cases. Even if they were workable, there should be clearer exceptions where a researcher has already relied on their consent. We are also concerned about whether amendments to the exemptions for HIPAA-regulated entities will make it harder for patients with rare diseases in Montana to secure access to genetic testing.

In practice, SB 351 would make Montana uniquely and particularly hostile to genetic research and innovation. It would mean that that patients with rare diseases could have an even harder time finding appropriate clinical care, and it would limit research that is badly needed to save lives. In the rare disease space, patients, families, researchers, and industry are aligned to further innovation and research while maintaining patient privacy. Yet the provisions of SB 351 do just the opposite. They limit genetic research that could lead to new therapies and cures benefiting individuals with rare diseases, and, at the same time, they fail to increase privacy protections in a meaningful way.

We appreciate the opportunity to share background on these important issues, and we are happy to answer any questions and work with you to modify the legislation which would advance research for all populations. Please feel free to contact me at 202-631-5752 or by email at mike@rareaccessactionproject.com.

Sincerely,

A handwritten signature in black ink, appearing to read "Michael Eging". The signature is fluid and cursive, with a large loop at the end.

Michael Eging
Executive Director
Rare Access Action Project

⁴ *Id.*