April 29, 2022

Dear Rare Families and Our Shared Community,

On Wednesday Sio Gene Therapies, Inc. shared the news of the company's decision to terminate its licensing agreement for GM1 gangliosidosis and GM2 (Tay-Sachs and Sandhoff diseases) gene therapies with the University of Massachusetts Chan Medical School. While this news is upsetting to all, we as patient advocacy organizations and foundations along with our partnering clinicians and researchers, are united and resolute in our commitment to advancing treatments including gene therapies for GM1 and GM2. Leaders from our six organizations and foundations met yesterday, and we will continue to meet as we identify next steps and the path forward.

As a rare community, we have faced challenges before, *and* at the same time we have come a long way. In the case of Sio's clinical trials for GM1 and GM2, the research data has been consistently positive and promising. Furthermore, the trials remain in good standing with the U.S. Food and Drug Administration (FDA). The reason for termination is financial. Like many startup companies, Sio relies on investors to underwrite the expensive costs of gene therapy. During this heightened uncertain economic climate, entire sectors including biotech have been affected.

We regret that we do not have more information at this time, but we pledge to share information as soon as we do. In the meantime, if you or your family need support, please reach out to us.

The fight continues.

Sincerely,

Nicola Dent
Executive Director
Blu Genes Foundation

Daniel and Patricia Lewi
Founders
Cure & Action for Tay-Sachs Foundation (CATS)

Christine Waggoner
President, Co-Founder
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