

Update on Spark Therapeutics Gene Therapy Program in Pompe Disease

Dear Pompe Community,

We are disappointed to share that, after careful consideration, Spark® Therapeutics has decided to discontinue our gene therapy program in Pompe disease following the pause of the program in 2022.

The decision to close our Pompe program was not related to any safety issues or concerns. Rather, the decision was part of a larger portfolio review and realignment of our strategy as an organization to ensure we're delivering impactful gene therapies to people as quickly as possible.

We recognize that this may be difficult news, and want to first thank the Pompe community for your enthusiasm and support over the last six years. We are deeply grateful to the study participants who were dosed in RESOLUTE, and will provide the follow-up and support required as part of the study protocol.

Additionally, we want to thank the many Patient Advocacy organizations for their collaboration, including the International Pompe Association, the Acid Maltase Deficiency Association, the Canadian Association of Pompe, Pompe Deutschland, Associazione Italiana di Miologia, Pompe Support Network, Association Francophone des Glycogénoses, Pompe Alliance, Associazione Italiana Glicogenosi, United Pompe Foundation, Asociación Española de Enfermos de Pompe, New Zealand Pompe Network, Australian Pompe Association, and the Association for Glycogen Storage Disease (UK).

If you have any questions about this update, please do not hesitate to contact us at <https://sparktx.com/contact-us/>. For study participants and their family members, we encourage them to reach out to their study physician for more information.

With Gratitude,

Jacose Bell - Pompe Patient Advocacy

Karen Gallagher – Pompe Asset General Manager

