London, May 19th, 2023

Dear Members of the Niemann-Pick UK Community,

As the co-founder & CEO of Bloomsbury Genetic Therapies Limited ("Bloomsbury"), I would like to take this opportunity to introduce Bloomsbury to you and to share our plans and progress in developing a new gene therapy treatment for Niemann-Pick type C disease ("NPC").

Bloomsbury is a clinical-stage biotechnology company that is dedicated to developing potentially curative treatments for rare neurological and metabolic diseases, including NPC. We are a spin-out from University College London (UCL) and commenced operations in 2022. We are underpinned by world-leading gene therapy and rare disease expertise from our academic founders from UCL, Professors Paul Gissen, Manju Kurian, Ahad Rahim and Simon Waddington. Our approach is to leverage de-risked, clinically proven gene therapy platforms to maximise therapeutic safety and efficacy, enable high manufacturability and accelerate the timeline to regulatory approval.

One of the gene therapy research programs that we are advancing is BGT-NPC, a highly differentiated gene therapy for the treatment of NPC. BGT-NPC is a neuron-targeted adeno-associated virus-based (or AAV) investigational gene therapy that aims to restore functional expression of the *NPC1* gene following a one-time injection in the brain. The program is currently in preclinical development and has already demonstrated compelling preclinical efficacy, and we aim to complete comprehensive preclinical safety and efficacy studies in the next two to three years. Our collaborators from UCL have just shared this week some of the latest data we have on the program at the ASGCT congress in the US, and they are preparing a publication in a scientific journal. Given the rarity of the disease and the urgent unmet medical need, once we have completed our planned preclinical activities, we intend to work with the regulators to design a clinical development plan based on a single phase 1/2/3 clinical trial, which we believe would enable an accelerated timeline towards regulatory approval and, ultimately, patient access.

We are currently seeking additional funding from investors and other sources to support the timely development of our programs, including BGT-NPC. We believe these programs have the potential to transform the lives of patients with rare neurological and metabolic diseases and it is a privilege to have been able to share our progress with you.

To learn more about us and our work, and to stay updated on our latest developments, please visit our website (https://bloomsburygtx.com) or our LinkedIn page (https://www.linkedin.com/company/bloomsburygtx/).

All the best to you and your families,

Adrien Lemoine