Bloomsbury Genetic Therapies Receives Orphan Drug Designations from the European Commission and the US FDA for BGT-NPC for the Treatment of Niemann-Pick Disease Type C and Provides Update on Type B Pre-IND Interaction with the US FDA

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– Type B pre-IND interaction with the US FDA provides constructive feedback on the Company's CMC, non-clinical and clinical development plans for BGT-NPC –

London, UK, 17 October 2023 – Bloomsbury Genetic Therapies Limited, a clinical-stage biotechnology company developing potentially curative treatments for patients suffering from rare neurological and metabolic diseases based on clinically proven gene therapy technologies, announced today that the Company has received Orphan Drug Designations (ODD) from the US Food and Drug Administration (FDA) and the European Commission (EC) for BGT-NPC, an investigational gene therapy for the treatment of Niemann-Pick Disease Type C (NPC).

The Company also received a detailed Type B Pre-Investigational New Drug Application (IND) Written Response from the FDA, providing constructive feedback on the Company's plans for chemistry, manufacturing, and controls (CMC), non-clinical and clinical development for BGT-NPC. Following the FDA feedback and the UK Medicines and Healthcare Products Regulatory Agency (MHRA) feedback disclosed in June 2023, the Company plans to initiate a toxicology and biodistribution study in rodents ahead of a single, multi-centric Phase 1/2/3 clinical trial. The Company is currently completing preclinical studies for BGT-NPC in collaboration with its partner University College London (UCL).

"These Orphan Drug Designations recognise the significant unmet need in patients in the United States and the European Union living with Niemann-Pick Disease Type C, a devastating disease with poor prognosis," said Adrien Lemoine, Co-Founder & Chief Executive Officer of Bloomsbury. "We are very encouraged by the outcome of our pre-IND interaction with the FDA, and we look forward to an ongoing, constructive dialogue with the FDA as we progress our development plans for BGT-NPC in the US."

In the US, the FDA grants ODD to a drug or biologic intended to treat a rare disease or condition, which generally includes a disease or condition that affects fewer than 200,000 individuals in the

US. ODD provides opportunities for grant funding towards clinical trial costs, tax advantages, FDA user-fee benefits, and seven years of market exclusivity in the US in the event of regulatory approval.

Similarly, in the EU, ODD is granted by the EC based on a positive opinion issued by the EMA Committee for Orphan Medicinal Products (COMP). It is intended to encourage the development of drugs that may provide significant benefit to patients suffering from rare, life-threatening diseases. This designation provides special incentives for sponsors, including eligibility for protocol assistance and possible exemptions or reductions in certain regulatory fees. In addition, if approved for marketing, this designation will provide ten years of marketing exclusivity.

– ENDS –

Enquiries
JW Communications
Julia Wilson
Tel: +44 (0)7818 430877

About Bloomsbury

Bloomsbury is a clinical-stage biotechnology company, developing potentially curative treatments for patients suffering from rare neurological and metabolic diseases based on clinically proven gene therapy technologies. The Company was spun out of University College London and launched in October 2022 with funding from UCL Technology Fund. Bloomsbury is building a pipeline of highly differentiated first- or best-in-class programs. For more information, please visit www.bloomsburygtx.com

About BGT-NPC

BGT-NPC is an investigational AAV9 gene therapy designed to provide a potentially curative solution to NPC patients following a one-time injection in the cerebrospinal fluid (CSF). BGT-NPC is currently completing preclinical studies, with compelling preclinical efficacy data already demonstrated. BGT-NPC has been granted Orphan Drug Designation (ODD) for the treatment of NPC by the European Commission (EC) and the US Food and Drug Administration (FDA), as well as Rare Pediatric Disease Designation (RPDD) from the FDA.

About Niemann-Pick Disease Type C

Niemann-Pick Disease Type C (NPC) is a rare, inherited and fatal neurodegenerative disease. 95% of NPC cases are caused by mutations in the *NPC1* gene that encodes for NPC1, a large protein which is embedded in the lysosomal membrane. When it is mutated or absent, both cholesterol and sphingolipids accumulate in the brain, liver, lungs, bone marrow, and spleen. Sphingolipids are

known to play important roles in signal transduction and nerve-fibre insulation, and their abnormal storage leads to irreversible neurological damage. There are several disease subtypes which are categorised by the age of onset of neurological disease. The patients develop progressive disabilities such as impairment in swallowing and speech, epilepsy, cerebellar ataxia (an inability to coordinate balance, gait, extremity and eye movements), and progressive dementia. The speed of progression of symptoms depends on the age of onset, which ranges from just after birth through to late adulthood. There are no approved treatments for NPC in the US. Miglustat is approved in the EU, but its use only slows disease progression. There are no curative treatments currently approved for NPC and there is a significant need to improve the standard of care. The incidence of NPC is estimated to be ~1:100,000 live births worldwide.