MEDIA RELEASE

Lonza Supporting Rocket Pharma with Clinical Manufacturing of LAD-I for the Treatment of Leukocyte Adhesion Deficiency-I.

- Clinical manufacturing to be carried out at Lonza's Houston (TX), USA and Geleen, Netherlands cGMP manufacturing sites for supply in the USA and in Europe
- Ongoing collaboration and tech transfers started in 2018 for the clinical manufacturing of LAD-I
- Announcement of collaboration is made public following Rocket Pharma's recent publication of positive Preliminary Data from Phase 1/2 Trial of RP-L201 for Leukocyte Adhesion Deficiency-I

Quote from Alberto Santagostino, SVP Head of Cell & Gene Technologies, Lonza Pharma & Biotech:

"We are firm believers and supporters of the science developed by Rocket Pharma. Our close collaboration with this team started over two years ago and has enabled the delivery of life-saving treatments to young patients left with no other options. With the presentation of the preliminary data from the first of these patients, Rocket Pharma is now demonstrating the potential of its novel platform technology to treat rare and devastating diseases successfully with gene therapies. We are fully committed to continuing to enable Rocket Pharma to deliver these treatments to many more patients in the US and in Europe, as they progress towards commercialization."

Basel, Switzerland, 28 January 2020 – Lonza today announced that a clinical manufacturing agreement is in place with Rocket Pharmaceuticals, Inc., a clinical-stage company advancing an integrated and sustainable pipeline of genetic therapies for rare childhood disorders. The agreement covers development and manufacturing for LAD-I, a phase 1/2 candidate for the treatment of Leukocyte Adhesion Deficiency-I (LAD-I).

The companies have been working together since 2018 and decided to make the collaboration public in parallel to the successful Preliminary Data from Phase 1/2 Trial announced by Rocket on December 9th, 2019.

The agreement covers the manufacturing of clinical product leveraging Lonza's lentiviral vector platform. Manufacturing takes place in both the Houston (TX), USA and Geleen, Netherlands Lonza cGMP manufacturing sites. The agreement also includes analytical assays and development services.

Autologous hematopoietic stem and progenitor cells (HSPCs) are transduced *ex vivo* with a lentiviral vector (LV) containing the *ITGB2* gene encoding for the human CD18 receptor (β2 integrin subunit). The therapy aims to treat Leukocyte Adhesion Deficiency-I (LAD-I). LAD-I is a rare immune disorder characterized by low or absent neutrophil CD18 expression, predisposing affected individuals to recurrent and fatal infections in childhood. Initial results from the first patient treated with RP-L201 demonstrated early evidence of safety and potential efficacy.

About Lonza

Lonza is a leading global supplier to the pharmaceutical, biotech and specialty ingredients markets. We work to promote a healthier lifestyle and prevent illness by supporting our customers to deliver innovative medicines that help treat or even cure a wide range of diseases. This is complemented by our broad range of microbial control solutions, which help to create and maintain a healthy environment.

Patients and consumers benefit from how we apply our scientific knowledge and advanced manufacturing technologies to the healthcare, hygiene and fast-moving consumer goods markets and to developing preservation and protection materials.

Founded in 1897 in the Swiss Alps, Lonza today is a well-respected global company with more than 100 sites and offices and approximately 15,500 full-time employees worldwide (at the end of 2019). The company generated sales of CHF 5.9 billion in 2019 with a CORE EBITDA of CHF 1.6 billion. Find out more at www.lonza.com.

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