OUR APPROACH

# The plato™ Platform

**ONE PLATFORM designed for state-of-the-art gene therapy**

We have spent years optimizing the elements of plato™, our proprietary gene therapy platform. It is designed as a foundation for potentially scaling gene therapies worldwide.

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## What is plato™?

The plato™ platform includes:

A lentiviral vector and proprietary tags designed to optimize vector copy number, transduction efficiency and resulting enzyme/protein activity in target cells.

Advanced cryopreservation to extend shelf life and enable flexible scheduling for patients and healthcare providers.

A personalized conditioning regimen with precision dosing through the use of therapeutic drug monitoring.

An automated, closed manufacturing process intended to improve consistency and predictability of the drug product.

The system is self-contained and compact, which we believe will facilitate expansion of global manufacturing sites.

## Why lentiviral vectors?

We use lentiviral vectors to integrate the therapeutic gene into the patient's own stem cells for several reasons:

- They have sizable cargo space, which allows us to insert even large therapeutic genes into the patient's stem cells.
- They are capable of permanently integrating a therapeutic gene directly into the patient's chromosomes.
  - This means when the patient's treated cells divide, each daughter cell is expected to carry the therapeutic gene and be capable of producing the functional protein the patient needs.

The process of integrating the therapeutic gene into the cells takes place in the lab, rather than in the patient's body. This approach is called *ex vivo* gene therapy.

It's important to note that our therapies are still investigational; they have not been approved by any regulatory body. However, they are based on more than 30 years of industry research into *ex vivo* gene therapy, including successful treatments of dozens of children and adults with thalessemia. Lentiviral vectors have also been studied extensively and used in the clinic with more than 350 patients undergoing *ex vivo* gene therapy over the last decade.