



Joint analyst call: Orchard Therapeutics & Pharming Group

Dr Sijmen de Vries, CEO, Pharming Group
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Forward-looking Statements



This presentation may contain forward-looking statements. Forward-looking statements are neither historical facts nor assurances of future performance. Instead, they are based on our current beliefs, expectations and assumptions regarding the future of our business, future plans and strategies, our development plans, our clinical results and other future conditions. All statements other than statements of historical facts contained in this presentation, including statements regarding our future financial or business performance, conditions, plans, prospects, trends or strategies, objectives of management and other financial and business matters; our current and prospective product candidates, planned clinical trials and preclinical studies, projected research and development costs, current and prospective collaborations; and the estimated size of the market for our product candidates, the timing and success of our development and commercialization of our product candidates and the market acceptance thereof, are forward-looking statements. New risks and uncertainties may emerge from time to time, and it is not possible to predict all risks and uncertainties. While we may elect to update these forward-looking statements at some point in the future, we assume no obligation to update or revise any forward-looking statements except to the extent required by applicable law. Although we believe the expectations reflected in such forward-looking statements are reasonable, we can give no assurance that such expectations will prove to be correct. Accordingly, readers are cautioned not to place undue reliance on these forward-looking statements. No representations or warranties (expressed or implied) are made about the accuracy of any such forward-looking statements.

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On the Call Today



Dr Sijmen de Vries
CEO, Pharming Group



Dr Anurag Relan
CMO, Pharming Group



Dr Bobby Gaspar
CEO, Orchard Therapeutics

Company Overview: Pharming Group



- ❖ Established in 1988, based in the Netherlands with 250+ employees
- ❖ Listed on the Nasdaq: **PHAR** & Amsterdam stock exchange: **PHARM**
- ❖ **Rare and ultra-rare disease development and commercialisation:**
 - Marketed lead product: **RUCONEST® (rhC1INH)**
 - Recombinant human C1-esterase inhibitor (enzyme replacement therapy) developed using our unique technology platform
 - Approved for the treatment of acute angioedema attacks in patients with hereditary angioedema (HAE)
 - Established commercial infrastructure in the USA and EU, and in partnership in Latin America, Korea and Israel
 - Clinical trials in follow-on indications
- ❖ Late-stage in-licenced product: **leniolisib**, for the treatment of Activated Phosphoinositide 3-kinase Delta Syndrome (APDS)

Three-pillar strategy for growth

Continuing to grow RUCONEST® sales through further country launches & increasing HAE market share

- Fully commercialize RUCONEST® in all major international markets with our own sales forces
- Improve convenience of therapy for HAE patients
- Evaluate new technologies to treat HAE



Grow our HAE franchise

Expanding indications for rhC1INH & developing new recombinant proteins using our platform technology

- Developing rhC1INH for additional large unmet indications
- Leverage our transgenic manufacturing technology to develop next-generation protein replacement therapies



Extend rhC1INH franchise to larger indications and develop new Enzyme Replacement Therapies

In-licensing or acquiring late-stage clinical development candidates

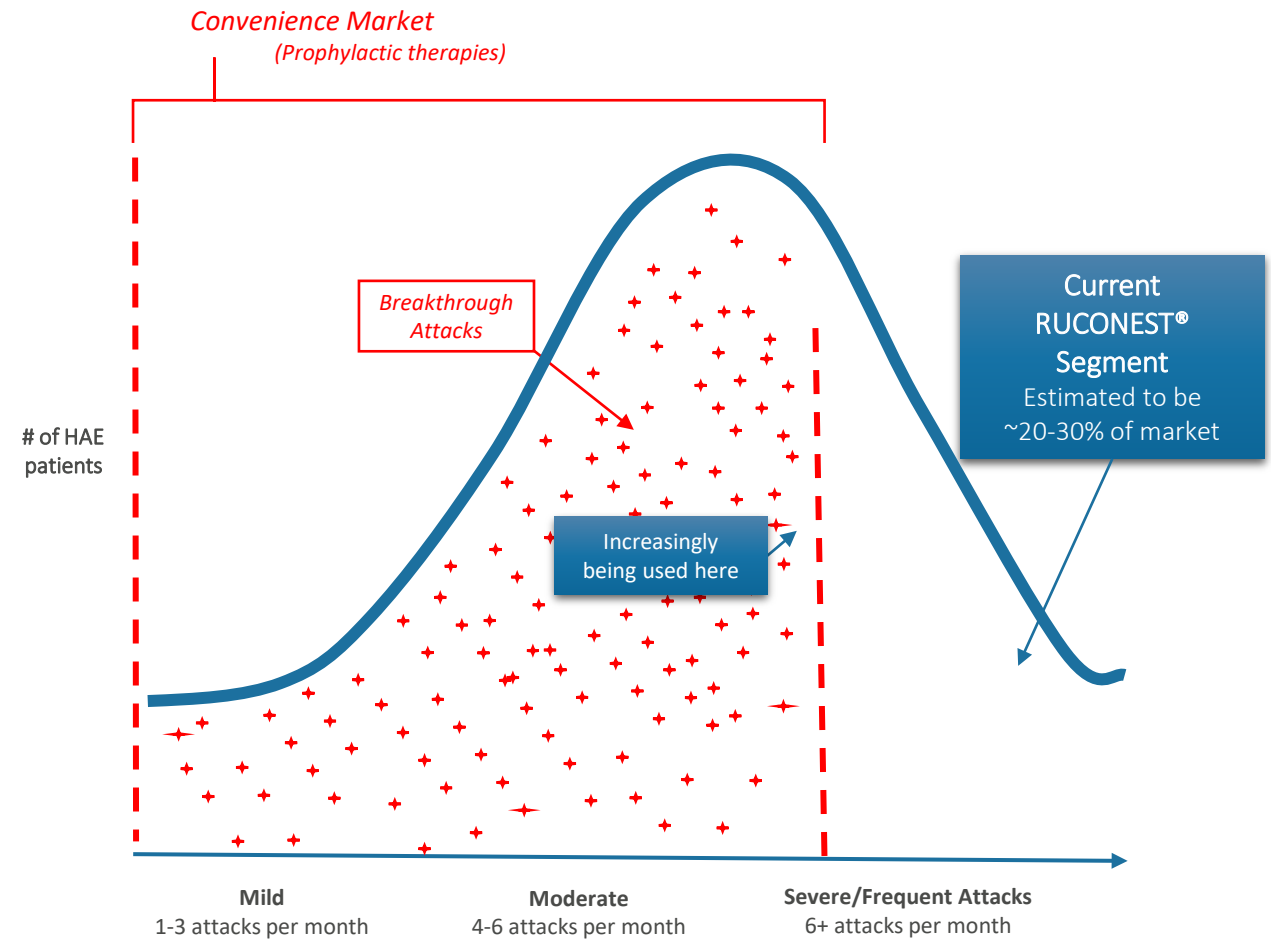
- Developing leniolisib for the treatment of APDS
- Developing or acquiring new programs or companies that can be commercialized using our sales and marketing infrastructure



Leverage commercial infrastructures and accelerate expansion of portfolio

Hereditary Angioedema

- HAE is caused by a deficiency of C1-INH, resulting in attacks of severe swelling (angioedema) in various parts of the body
- Patients use medication for treatment and prevention (prophylaxis) of attacks
 - RUCONEST® approved for the treatment of acute HAE attacks in adults and adolescents in the US and EU
- Increasing use of prophylaxis because patients want to be attack-free
 - New treatments offer better attack reduction rates than previous IV plasma-derived C1INH prophylaxis treatment
 - Although kallikrein/bradykinin inhibitors block the main pathway for symptomatology, C1-INH levels remain low
 - ~ Half of the patients using new prophylaxis treatments continue to have breakthrough attacks, some frequently, and are in need of regular use of breakthrough medication



Forward-looking Statements



Certain information set forth in this presentation and in statements made orally during this presentation contains “forward-looking statements”. Except for statements of historical fact, information contained herein constitutes forward-looking statements and may include, but is not limited to, the Company’s expectations regarding: (I) the safety and efficacy of Libmeldy and its product candidates; (II) the expected development of the Company’s business and product candidates, including in regards to larger indications; (III) the timing of regulatory submissions for approval of its product candidates; (IV) the timing of interactions with regulators and regulatory submissions related to ongoing and new clinical trials for its product candidates; (V) the timing of announcement of preclinical and clinical data for its product candidates and the likelihood that such data will be positive and support further development and regulatory approval of these product candidates; (VI) the timing and likelihood of approval of such product candidates by the applicable regulatory authorities; (VII) execution of the Company’s vision and growth strategy, including with respect to global growth and in larger indications; (IX) the size and value of potential markets for the Company’s product candidates; and (X) projected financial performance and financial condition, including the sufficiency of the Company’s cash and cash equivalents to fund operations in future periods and future liquidity, working capital and capital requirements. The words “may,” “should,” “expects,” “intends,” “plans,” “anticipates,” “believes,” “estimates,” “predicts,” “potential,” “continue,” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Forward-looking statements are provided to allow investors the opportunity to understand management’s beliefs and opinions in respect of the future so that they may use such beliefs and opinions as one factor in evaluating an investment.

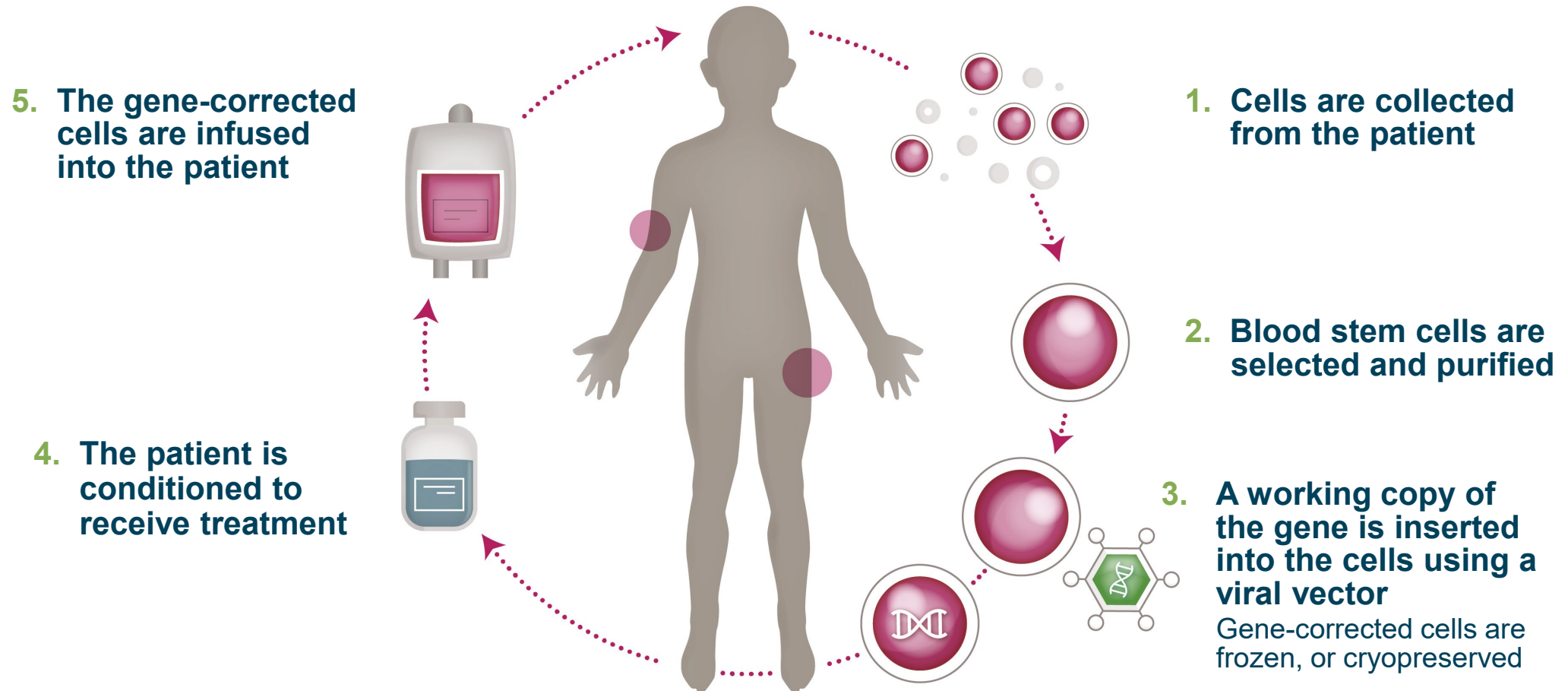
These statements are neither promises nor guarantees of future performance. Such forward-looking statements necessarily involve known and unknown risks and uncertainties, which include, without limitation, the severity of the impact of the COVID-19 pandemic on the Company’s business, including on preclinical and clinical development and commercial programs, which may cause actual performance and financial results in future periods to differ materially from any projections of future performance or results expressed or implied by such forward-looking statements. You are cautioned not to place undue reliance on forward-looking statements. These statements are subject to a variety of risks and uncertainties, many of which are beyond the Company’s control, which could cause actual results to differ materially from those contemplated in these forward-looking statements. For additional disclosure regarding these and other risks faced by the Company, see the disclosure contained in the Company’s public filings with the U.S. Securities and Exchange Commission (the “SEC”), including in the Company’s quarterly report on Form 10-Q for the quarter ended March 31, 2021, as well as subsequent filings and reports filed with the SEC. These forward-looking statements speak only as of the date of this presentation. The Company undertakes no obligation to publicly update or revise any forward-looking statements, whether as a result of new information, future events or otherwise, except as may be required by law.

*The potential of
HSC gene therapy*

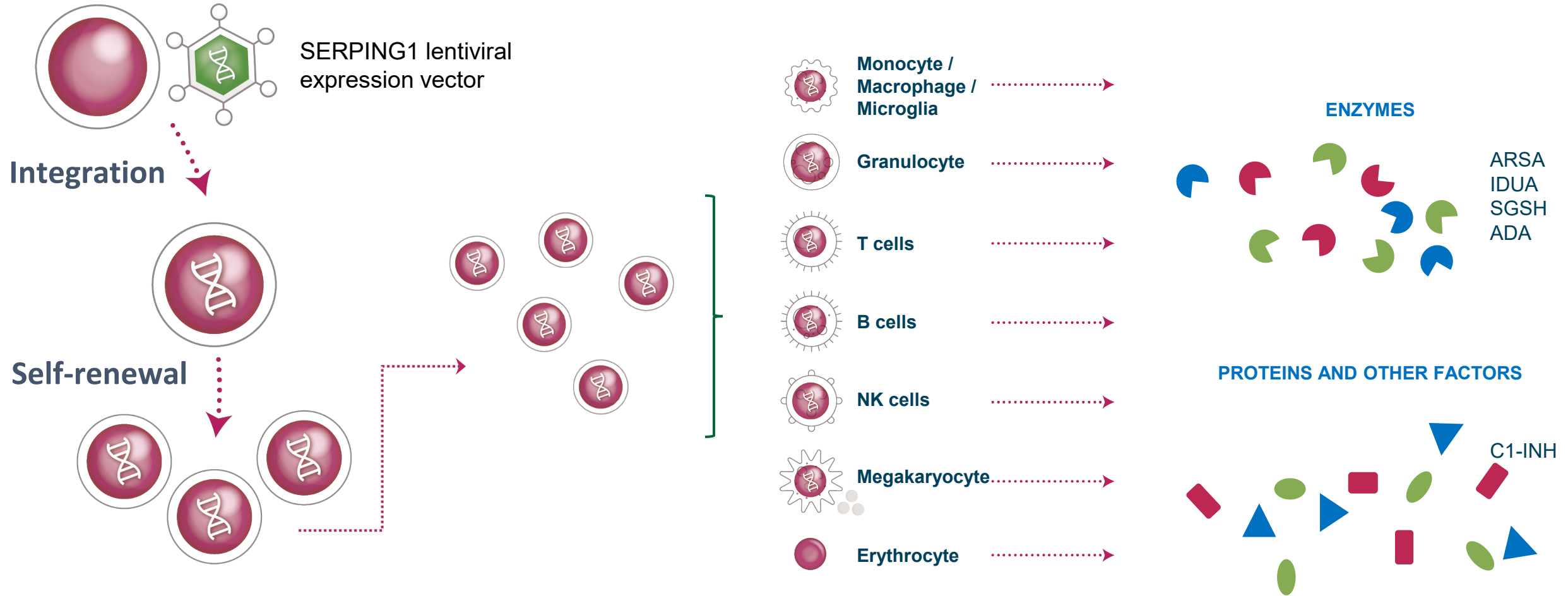


 Orchard
therapeutics™

The HSC Gene Therapy Approach



The Power of HSC Gene Therapy



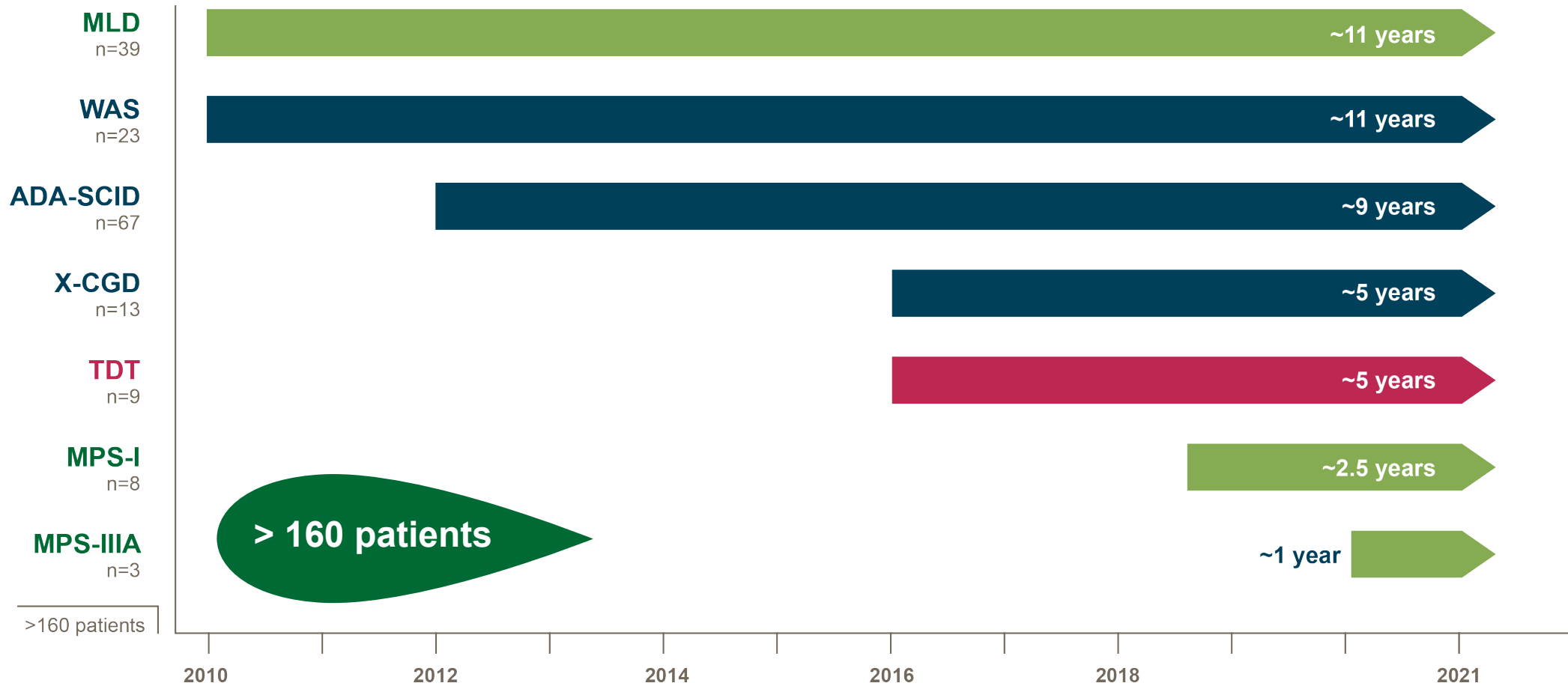
One-time
administration

Potential for permanent
disease correction

Differentiates into multiple
cell types

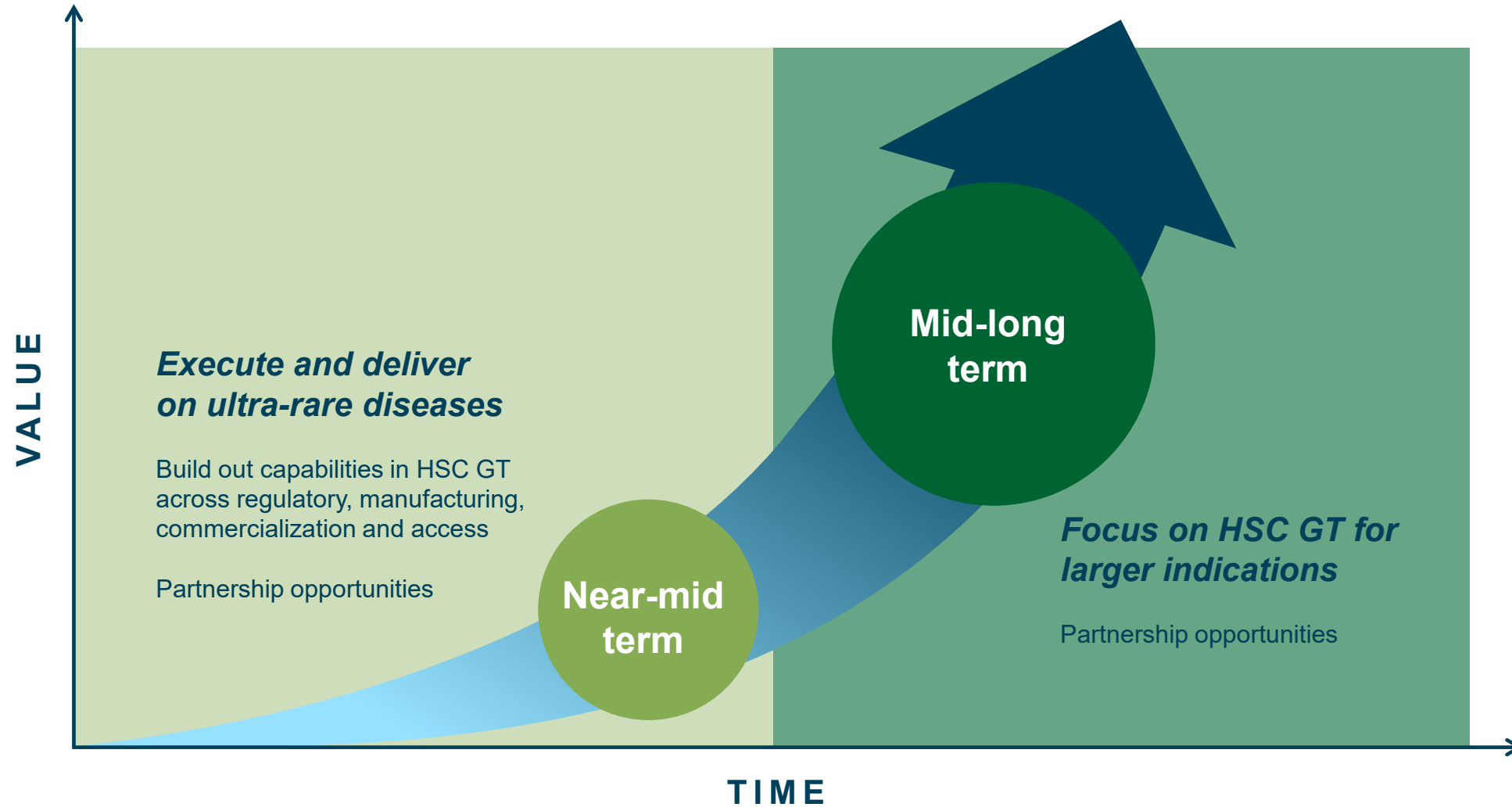
Enables secretion of specific
enzymes or factors

Durability of Response and Safety



Patients treated in the development phase, including in clinical trials and under pre-approval access (defined as any form of pre-approval treatment outside of a company-sponsored clinical trial, including, but not limited to, compassionate use, early access, hospital exemption or special license). Data based on in-house data as of February 2021. Data include all patients treated with CD34+ hematopoietic stem cells transduced ex vivo with vector of interest.

Evolving to Larger Indications



Driving Development of a Best-in-class HAE Gene Therapy



- Expertise in HSC gene therapy
- Vector development and testing
- Established CDMO network
- Murine transplant studies
- Internal discovery capabilities



- Extensive clinical and commercial expertise in HAE
- Pre-clinical disease models for HAE
- Capital to fund ongoing development

Together Orchard and Pharming can combine expertise and experience to develop a best-in-class HAE gene therapy to provide the potential for life-long prophylaxis following a single administration

\$17.5M

upfront payment (cash and equity)

Up to \$189.5M

in potential milestones

Mid-single to low double-digit

royalties on future sales

www.orchard-tx.com

Nasdaq: ORTX

www.pharming.com

ENXTAM: PHARM

Nasdaq: PHAR

Bloomberg: PHAR.AS