

A man in a red hoodie and grey pants is walking on a sandy path that overlooks a beach and rocky cliffs. The scene is bathed in warm, golden light, suggesting sunset or sunrise. The man is smiling and looking towards the right. In the background, there are waves crashing on a sandy beach, and the cliffs are rugged and yellowish-brown. A white rope barrier runs along the path. The overall mood is serene and scenic.

uniQure

Global License Agreement in Hemophilia B

June 24, 2020

This presentation contains forward-looking statements. All statements other than statements of historical fact are forward-looking statements, which are often indicated by terms such as “anticipate,” “believe,” “could,” “estimate,” “expect,” “goal,” “intend,” “look forward to,” “may,” “plan,” “potential,” “predict,” “project,” “should,” “will,” “would” and similar expressions. Forward-looking statements are based on management’s beliefs and assumptions and on information available to management only as of the date of this presentation. These forward-looking statements include, but are not limited to, statements regarding the development of our gene therapies, the success of our collaborations, and the risk of cessation, delay or lack of success of any of our ongoing or planned clinical studies and/or development of our product candidates. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with the impact of the ongoing COVID-19 pandemic on our Company and the wider economy and health care system, our collaboration arrangements, our and our collaborators’ clinical development activities, regulatory oversight, development of product candidates, product commercialization and intellectual property claims, as well as the risks, uncertainties and other factors described under the heading “Risk Factors” in uniQure’s Annual Report on Form 10-K filed on March 2, 2020 and Quarterly Report on Form 10-Q filed April 29, 2020. Given these risks, uncertainties and other factors, you should not place undue reliance on these forward-looking statements, and we assume no obligation to update these forward-looking statements, even if new information becomes available in the future.

- 1 **One of the largest gene therapy deals announced to date**
- 2 **CSL Behring is an ideal strategic partner with extensive commercial expertise in hemophilia that best positions uniQure to provide EtranaDez to the greatest number of global patients as quickly as possible**
- 3 **Strategically positions uniQure to focus on aggressively advancing its pipeline of gene therapy candidates, anchored by AMT-130 in Huntington's**
- 4 **~\$720M pro forma cash provides runway into 2H 2024 to fund pipeline expansion, invest in technology innovation and scale manufacturing capabilities**

- **The transaction is a historic collaboration in gene therapy and hemophilia, with uniQure eligible to receive more than \$2B in total economics**
 - \$450M upfront payment
 - \$1.6B in regulatory and commercial milestones
 - Double-digit royalty payments up to low-twenties percentage of net product sales
 - Reimbursement of uniQure's clinical and regulatory costs

- **Partnership enables uniQure to leverage CSL Behring's world-class global hemophilia commercial infrastructure**
 - Hemophilia is a well-established, specialized and highly competitive global market
 - CSL Behring has been a leader in bleeding disorders for more than 30 years
 - Deep, long-standing relationships with hemophilia communities worldwide
 - One of the broadest product portfolios in hematology and thrombosis
 - \$1B+ in hemophilia sales in 2019
 - Commercial sales in more than 100 countries

uniQure

Building Value Through Innovation



- **uniQure is a gene therapy pioneer with 20+-year history of industry-leading innovation across products and platform**
 - First approved gene therapy in the Western world
 - First AAV-delivered gene silencing therapy for Huntington's to enter clinic
 - First AAV vector that can be administered to patients with pre-existing NABs
 - 15 years of proven, commercial-scale cGMP manufacturing capabilities
 - Strong intellectual property portfolio covering both product and platform aspects
 - Next-gen portfolio of AAV capsids, promoters and other enabling technologies
 - World-class research and technical expertise

- **Ingenuity in practice – leapfrogging hemophilia B with a potentially first- and best-in-class gene therapy**
 - 2008: Licensed wtFIX gene cassette used by St. Jude in watershed first-in-human study
 - 2015: Initiated Ph I/II study of AMT-060 with wtFIX transgene
 - **2017:** *Announced transition to AMT-061 with AAV5/FIX-Padua transgene*
 - **2018:** *Initiated Ph IIb dose-confirmation study and Ph III pivotal study*
 - **2020:** *Completed dosing of 54 patients in Ph III pivotal study*

***Clinical and regulatory innovation:
uniQure completed dosing of its pivotal study just 2.5 years
after unveiling AMT-061***

1

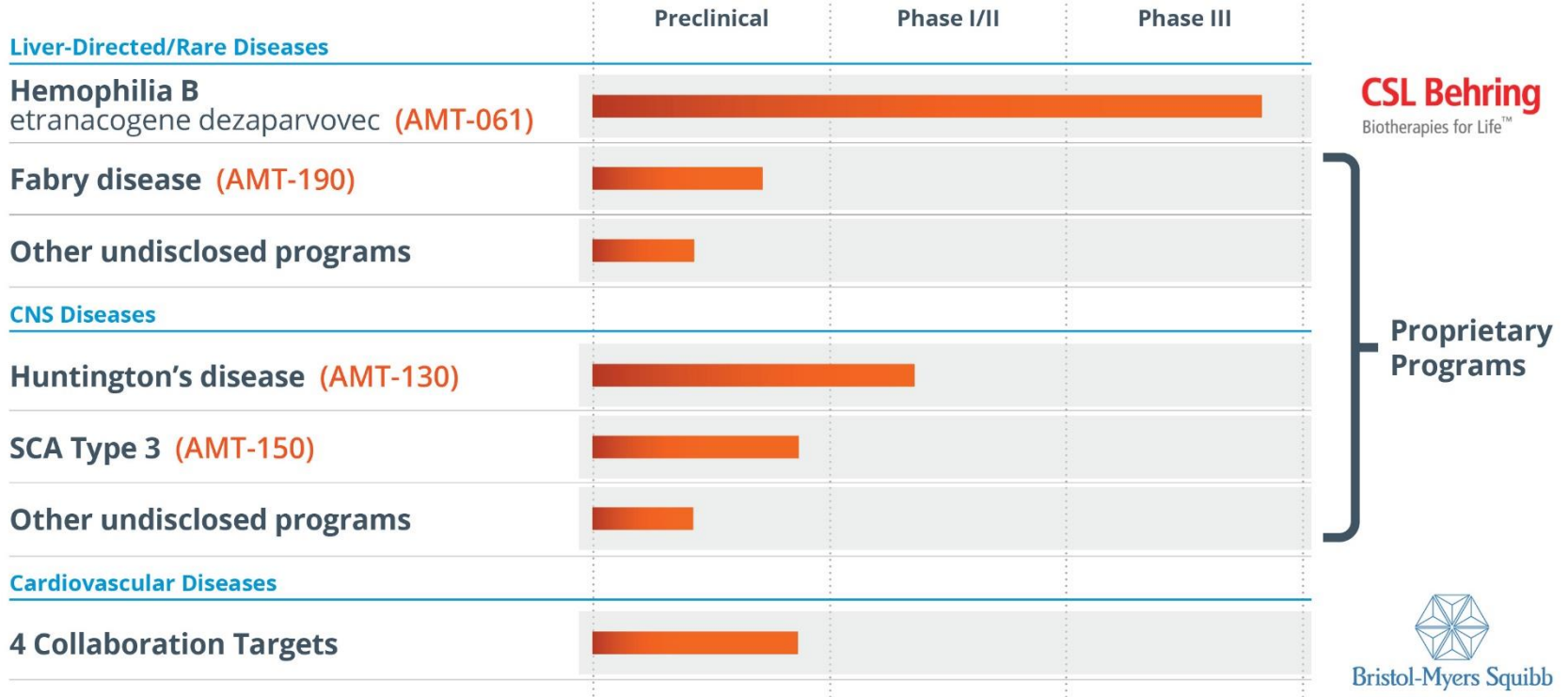
Aggressively Advance & Expand Gene Therapy Pipeline



2

Invest in and Strengthen Gene Therapy Platform







uniQure

Aggressively
Advance & Expand
Pipeline

1 Advance Huntington's Disease program

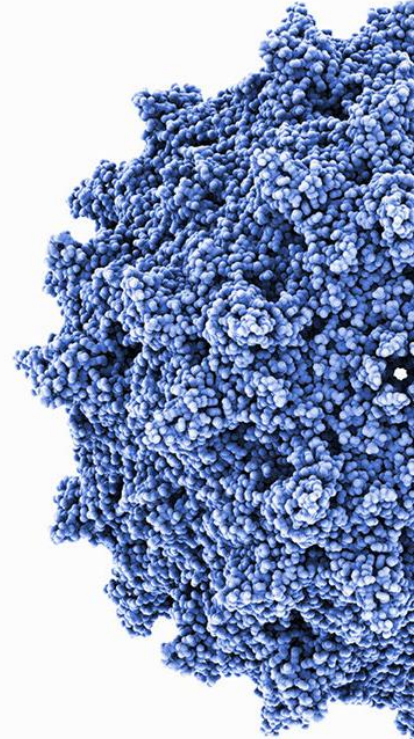
- Patient dosing initiated in June 2020 represents first-in-human AAV gene therapy for Huntington's
- Phase I/II safety data expected in 2020 with initial efficacy data expected in 2021
- Exploring next-generation, less-invasive administration technologies

2 Accelerate internal R&D leveraging both core and next-gen platforms

- Focus on CNS disorders and rare, liver-directed diseases
- AAV5 gene therapies – potentially more durable, less immunogenic, increased patient eligibility
- Next-generation gene therapies – potentially more targeted, effective and versatile

3 Expand in-licensing and/or acquisition of product candidates

- **Rapid path to IND and high regulatory familiarity**
- **Superior immunogenicity profile compared to other AAV vectors**
 - Potential to treat all or nearly all of patients
 - Potential for no immunosuppression
 - Potential for re-administration
- **Demonstrated clinical efficacy in liver-directed and CNS applications**
 - > 75 patients treated across 5 clinical studies
 - 8 years of patient follow-up
- **Proven capability to manufacture at large-scale**



uniQure

Invest in and
Strengthen Gene
Therapy Platform



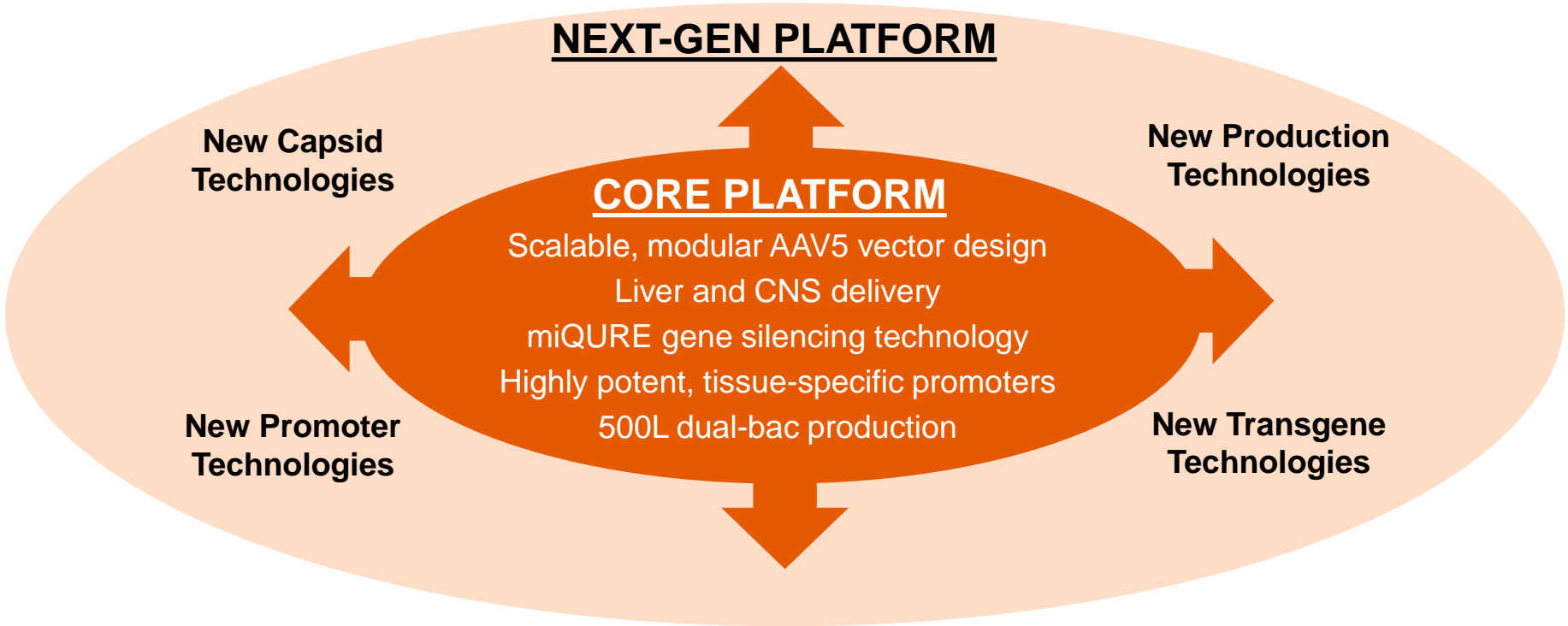
1 Build out organization to support expansion of pipeline

2 Further invest in new tools and new technologies to enable potential improvements in efficacy, safety and applicability of our gene therapy candidates

- Next-gen components (capsids, promoters, transgenes)
- Re-administration
- New modalities

3 Significantly expand manufacturing capacity and lower COGS to support larger indications and broader market access

- Scaling up and out
- Next-gen production technologies



Develop Potentially Best-in-Class Gene Therapies Through Innovation

Improved Targeting

More effective cell targeting, transduction and expression

- Nanobody-designed AAVs
- HDL-binding capsids
- GenX: Highly-specific, potent promoters and enhancers

Expanded Applications

Differentiated transgene technologies to broaden applicability of gene therapy

- miQure gene silencing platform
- Vectorized antibodies
- Substrate reduction

Robust Manufacturing

High-quality, large-scale and low-cost manufacturing

- Improved dual baculovirus platform
- Producer cell lines
- Improved yields, reduced variability and more flexible up-scaling

uniQure

Our 5-Year Vision



1 **Commercial-stage gene therapy company with best-in-class pipeline**

- 3 to 5 commercial/pivotal stage programs
- 5 to 10 Phase I/II programs

2 **Industry-leading manufacturing and technology capabilities**

- Lower-cost, 2,000L scale process capable of expanding to 10,000L
- Leading portfolio of next-gen capsids, promoters, transgenes and production technologies

3 **Ambition to directly commercialize Huntington's & other gene therapies**

uniQure