uniQure

Delivering Gene Therapy to Patients

Annual General Meeting of Shareholders

June 13, 2018

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Forward-looking Statements

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 press release. 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 collaboration arrangements, our and our collaborators’ clinical development activities, regulatory oversight, product commercialization and intellectual property claims, as well as the risks, uncertainties and other factors described under the heading "Risk Factors" in uniQure’s Quarterly Report on Form 10-Q filed on April 30, 2018. 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.

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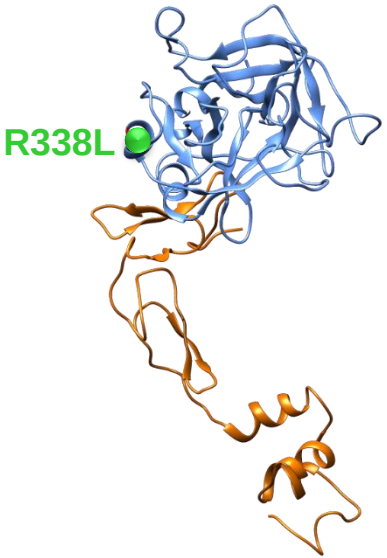
Recent progress

Execution across all programs

Hemophilia B (AMT-061)	• *Aligned with FDA to initiate dose-confirmation study* • *Preparing to initiate enrollment in lead-in phase of pivotal trial* • *Second Padua patent issued in U.S. covering methods of use*
Huntington's (AMT-130)	• *Positive new data presented in minipigs* • *Completed dosing of GLP toxicology study* • *Preparing for IND submission*
Heart Failure (AMT-126)	• *Initiated heart failure functional study in diseased minipig model* • *Ongoing progress in two additional programs*
Corporate	• *Successful $147.5 million capital raise* • *Share price +76% YTD*

AMT-061: potential best-in-class gene therapy

Padua: expresses a protein with a single amino acid substitution that has been shown to have a ~6 to 7-fold increase in FIX activity compared to the wild-type FIX protein



AMT-061: Differentiated Product Profile

- *Long-term safety, including favorable immunogenicity profile*
- *Predictable, sustained and potentially curative increases in FIX activity*
- *Significant reductions in bleeding rates and FIX replacement therapy*
- *Broad patient eligibility*

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AMT-061: pivotal study program

Dose-confirmation study:

- *~ 3 patients to receive a single dose of 2 x e^{13} gc/kg*
- *Short-term follow-up to observe FIX activity and confirm dose*
- *Will be conducted in parallel with patient enrollment into lead-in portion of pivotal study*

Pivotal study:

- *Open label, single-dose, multi-center, multi-national trial*
- *Approximately 50 patients with severe and moderately-severe hemophilia B*
- *Patients with AAV5 antibodies not intended to be excluded*
- *Patients will serve as their own control; 6-month lead-in to establish baseline*
- *Study objectives:*
  - *Increase FIX activity*
  - *Reduce frequency of bleeding episodes*
  - *Decrease use of FIX replacement therapy*
  - *Assess efficacy and safety*

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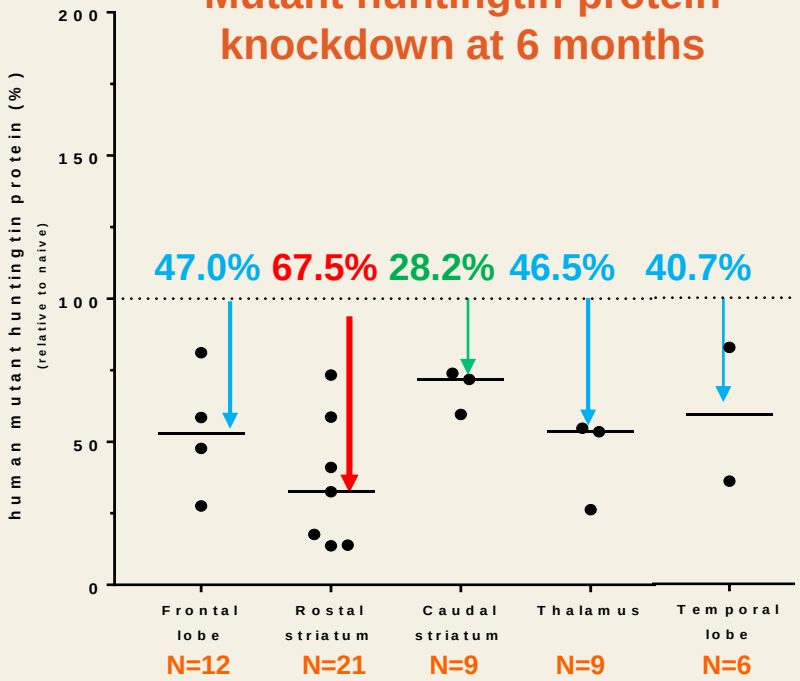
AMT-130: strong reduction of mHTT

Gene therapy for Huntington's disease

- *Non-selective* **knockdown of huntingtin protein**
- *Uses* **proprietary miRNA** *that degrades HTT mRNA*
- **One-time injection** *in the striatum*
- *AAV5 has* **widespread distribution in brain**
- **Demonstrated PoC** *in multiple animal models*
- **Same manufacturing platform** *as AMT-061*

Mutant huntingtin protein knockdown at 6 months



Median. Each dot represents the average value of 3 tgHD minipig animals

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AMT-130: status and next steps

Current Status

- *Established pre-clinical proof of concept*
- *Ongoing nonclinical safety toxicology studies*
- *Granted Orphan Drug Designation by FDA*
- *Granted Orphan Medicinal Drug Designation by EMA*

Next Steps

- *Submit IND for AMT-130 in 2H 2018*
- *Begin first-in-human clinical study*

Expanding our research pipeline

Research & Development Day

- *Late fall in New York City*
- *Introduce new programs/product candidates*
- *Highlight other novel enabling technologies*

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A strong financial position

US$ in millions*	2017	2016	2015
Revenue	*$13*	*$25*	*$11*
R&D	*$72*	*$73*	*$59*
SG&A	*$25*	*$26*	*$23*
Net Loss	*($79)*	*($73)*	*($82)*

US$ in millions*	As of 3/31/18
Cash	*$120*
Total Assets	*$170*
Debt	*$20*
Total Liabilities	*$124*
Shareholders' Equity	*$46*

- *Excludes gross proceeds of $147.5 million from May 2018 follow-on offering*
- *Current cash and cash equivalents expected to be sufficient to fund operations into 2021*

** In accordance with US GAAP*

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Near-term value drivers

Several upcoming milestones across all programs

Hemophilia B (AMT-061)	• *Initiate treatment of patients in dose-confirmation study* • *Presentation of FIX activity data from initial patients treated* • *Initiate enrollment in lead-in phase of pivotal trial*
Huntington's (AMT-130)	• *Complete IND-enabling GLP safety/tox study* • *Submit IND/CTA for Phase I/II study*
Heart Failure (AMT-126)	• *Complete heart function study of AMT-126 in diseased minipigs* • *Initiate IND-enabling GLP safety study*
Research	• *Presentation of new product candidates*

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