uniQure

Delivering Gene Therapy to Patients

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March 1, 2019

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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 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 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 February 28, 2019. 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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Strategic imperatives

Pipeline	*Develop a proprietary pipeline of gene therapy candidates focused on liver-directed and CNS disorders*
Manufacturing	*Maintain leadership in commercial-scale manufacturing of AAV gene therapies*
Enabling Technologies	*Invest and leverage next-generation technologies that optimize and expand the applicability of gene therapy to patients*
Intellectual Property	*Expand and maintain our leading IP portfolio*
Commercialization	*Retain valuable commercial rights*

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Expanding our proprietary pipeline…

*Research collaboration with Bristol-Myers Squibb

AMT-061: FIX activity up to 16 weeks post-treatment

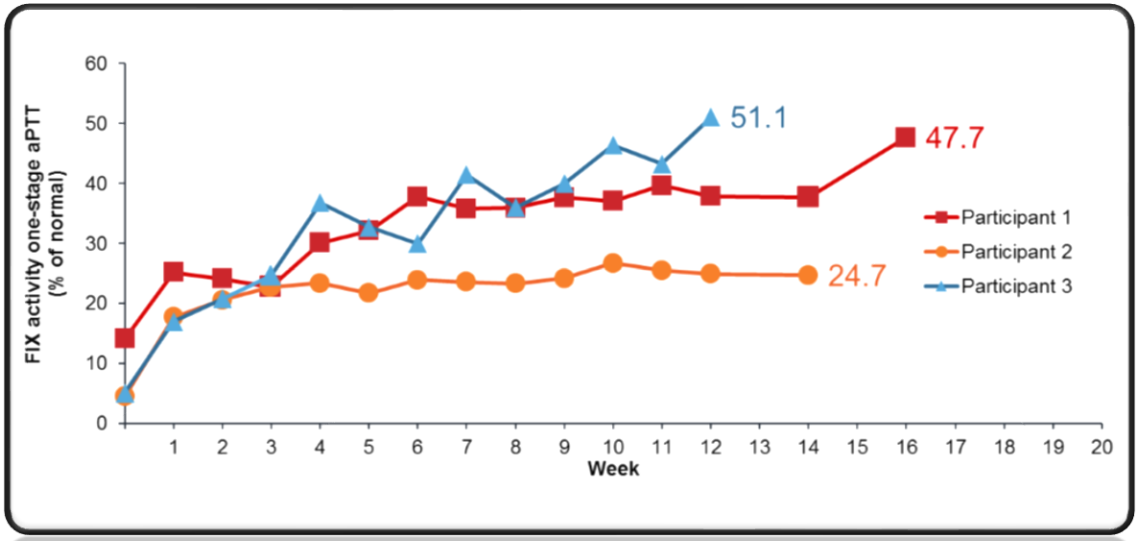
Main Efficacy Findings:

- ❑ *Sustained increases in FIX activity*
- ❑ *No bleeding events post-infusion*
- ❑ *No infusions of replacement therapy*
- ❑ *No requirement of immunosuppression*

Main Safety Findings:

- ❑ *Well-tolerated*
- ❑ *No serious adverse events*
- ❑ *No inhibitor development*

Increases in FIX Activity up to 51%
Mean FIX activity at 12 weeks of 38%



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AMT-061: HOPE-B Phase III 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 will not 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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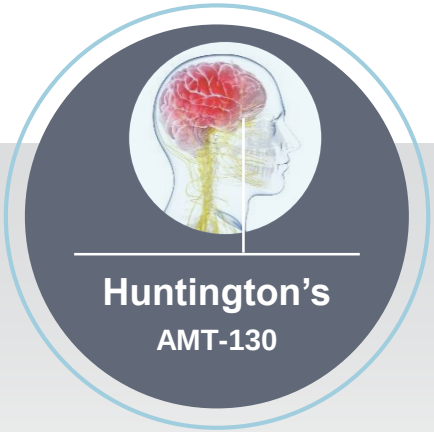
AMT-130: Huntington's Disease



- *3-7 per 100K people*[1]
- *No treatments available*
- *Strong preclinical data*
- *Near-term goal: Initiate clinical study in 2019*

Target product profile

- *One-time administration of disease-modifying therapy*
- *Proprietary miQURE™ silencing platform*
- *Strong mHTT knockdown in both deep structures and cortex*
- *Preclinically shown to be generally safe and well-tolerated*
- *Potential to be first to market*

[1] *Rawlins, MD. Neuroepidemiology 2016;46:144-153*

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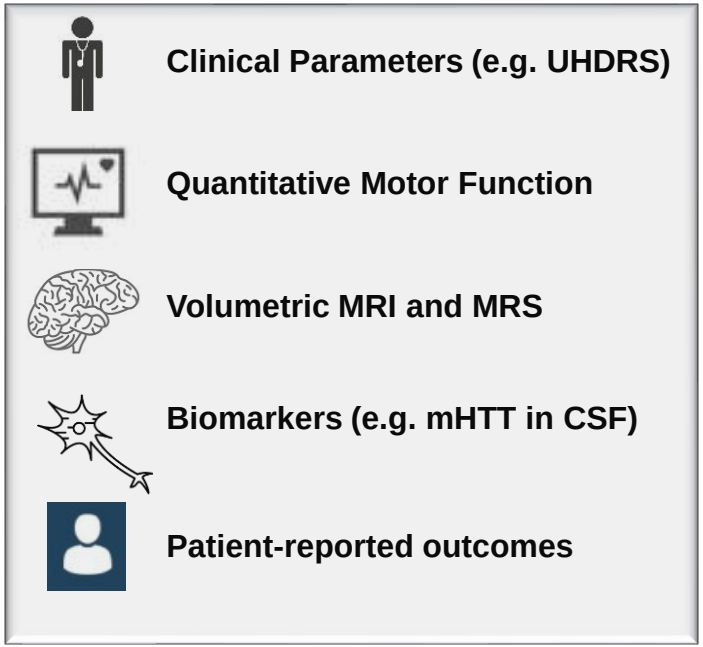
AMT-130: Phase I/II dose escalation study

IND Cleared – Phase I/II Study Overview

- *Multicenter, randomized, double-blinded study*
- *Placebo-controlled with imitation surgery*
- *Objectives: assess safety, tolerability and efficacy*
- *Two dose cohorts with a total of 25 patients*
- *18-month follow-up (5 years for treated patients)*

Efficacy Endpoints



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Near-term goals

Hemophilia B	**Complete enrollment in HOPE-B Phase III pivotal study of AMT-061**
Huntington's	**Initiate dosing of Phase I/II study of AMT-130**
Hemophilia A	**Submit IND for AMT-180**
Other Programs	**Initiate IND-enabling toxicology study for one additional program**

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