uniQure

Delivering Gene Therapy to Patients

Corporate Presentation

SEPTEMBER 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 August 8, 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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Near-term value drivers

Several milestones across key programs

Hemophilia B (AMT-061)	• Continue enrollment of AMT-061 pivotal trial • Treat ~3 patients to confirm dose of 2 x e^{13} gc/kg • Announce clinical data (FIX activity) from initial ~3 patients
Huntington's disease (AMT-130)	• Complete IND-enabling GLP safety study • Submit IND/CTA for Phase I/II study • Initiate Phase I/II study
Research Pipeline	• Complete heart function study of AMT-126 in diseased minipigs • Advance additional targets focused on liver/metabolic and CNS disorders • R&D Investor day in Q4 2018

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State-of-the-art manufacturing

Large-scale AAV Manufacturing Capability

- Based in Lexington, MA
- 3rd generation insect cell, baculovirus
- Scalable up to 2 x 2000L
- Ready for commercial scale-up

Benefits

- Control process through commercialization
- Highly scalable, cost-effective
- High-volume capacity
- Consistent, stable, high-quality products

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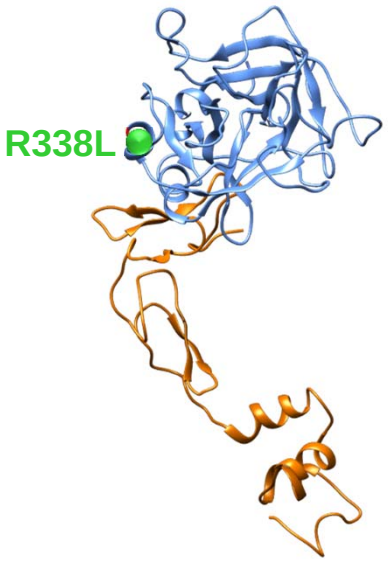
AMT-061: potential best-in-class gene therapy

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



Goal of hemophilia B Gene Therapy

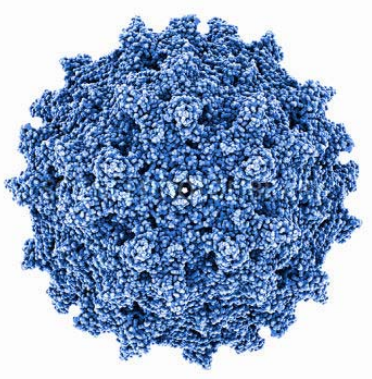
- Long-term safety, including favorable immunogenicity profile
- Predictable, sustained and potentially curative increases in FIX
- Significant reductions in bleeding rates/FIX replacement therapy
- Broad patient eligibility

AMT-061: leveraging AAV5's favorable immunogenicity profile

AAV5 – Clinically demonstrated tolerability and outcomes

- Data from 22 patients in 3 clinical studies[1]
- Demonstrated clinical outcomes in the liver and brain
- Lowest prevalence of pre-existing neutralizing antibodies[2]
- Favorable immunogenicity profile for systemic, intravenous delivery
- No confirmed T-cell-mediated immune responses to capsid

AAV5 Vector



[1] Clinical trials in Hemophilia B, Sanfilippo B and Acute Intermittent Porphyria
[2] Boutin et al 2014

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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-061: dose-confirmation study

- To be conducted in parallel with lead-in portion of pivotal study
- First patient treated as announced on August 23, 2018
- ~3 patients to receive a single dose of AMT-061 at $2e^{13}$ gc/kg
- ~6 to 8 weeks of follow-up to observe FIX activity and confirm dose
- Top line FIX data expected in 4Q 2018

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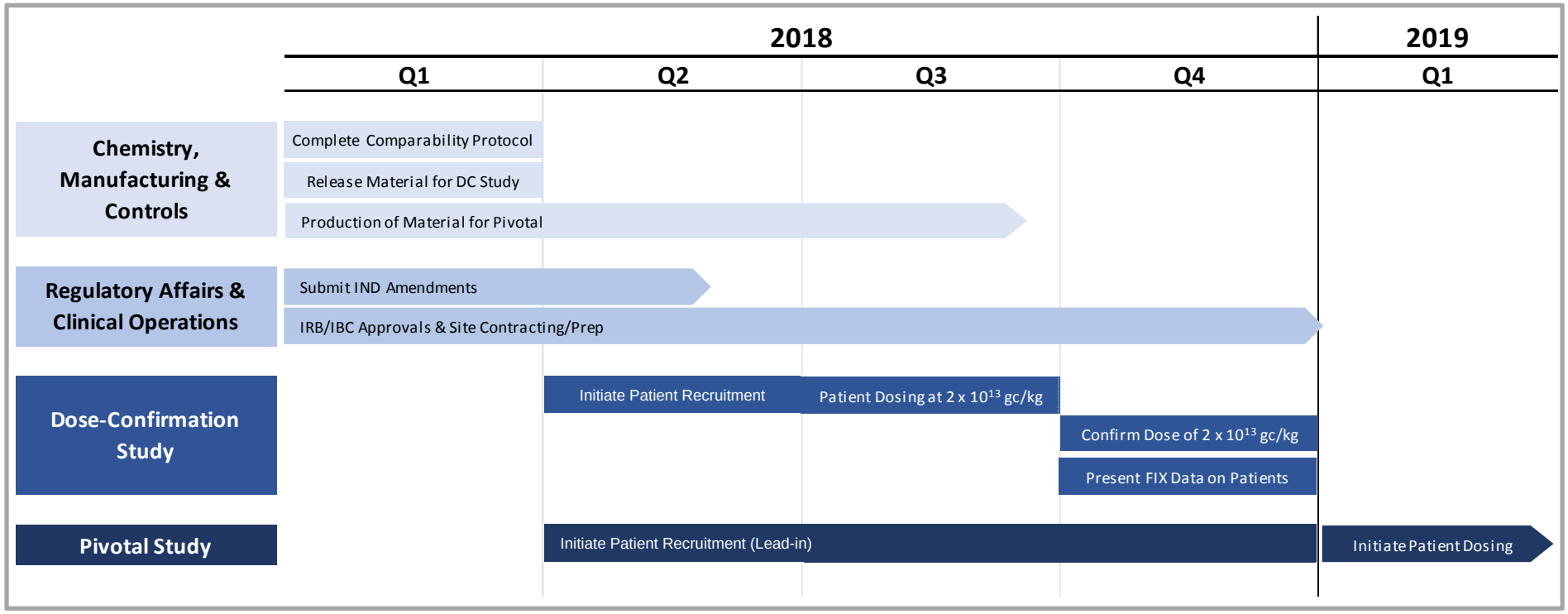
AMT-061: anticipated milestones



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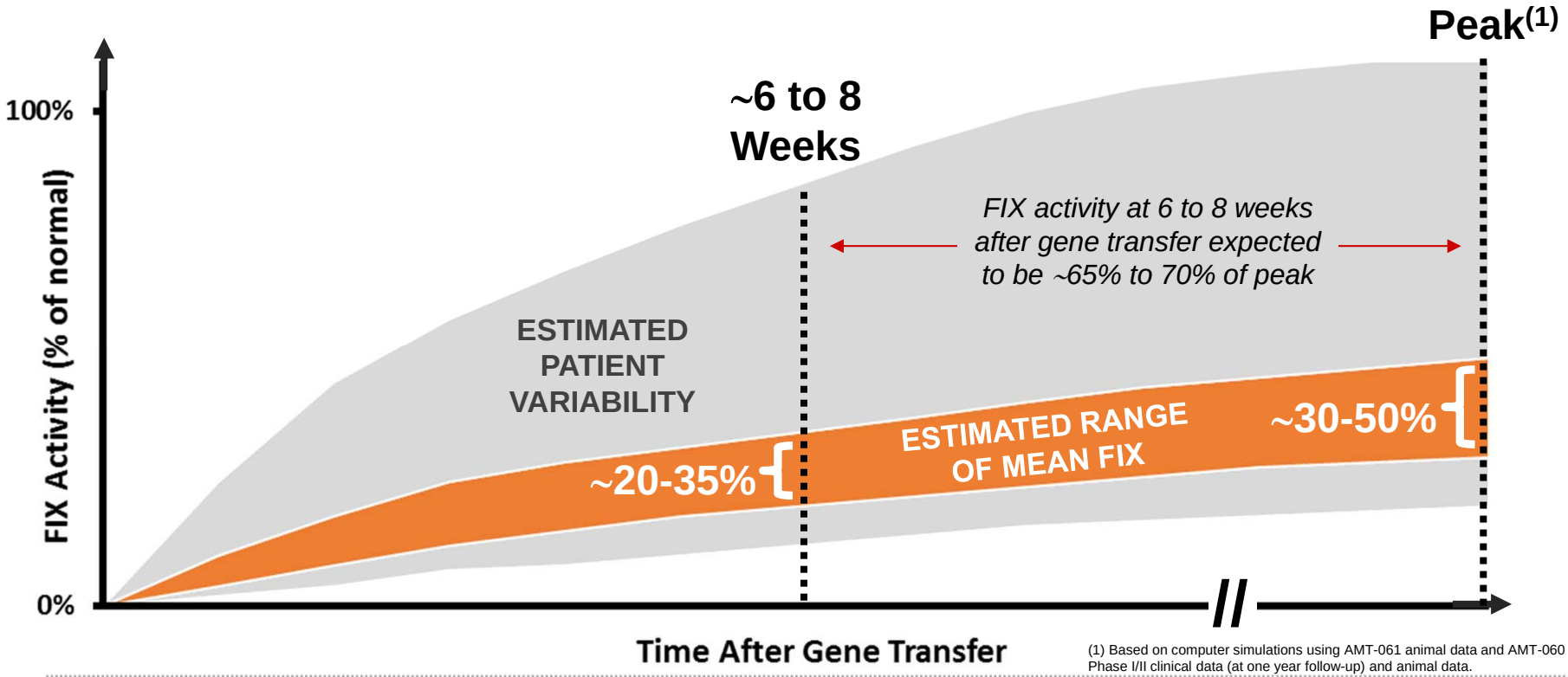
AMT-061: estimated FIX activity in humans



(1) Based on computer simulations using AMT-061 animal data and AMT-060 Phase I/II clinical data (at one year follow-up) and animal data.

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AMT-061: FIX-Padua intellectual property

- In 2017, acquired a patent family covering the FIX-Padua or the “Padua mutant”
  - Factor IX with a leucine at the R338 position of the protein sequence
- Second U.S. patent issued in April 2018 strengthens intellectual property position
  - Covers methods of treating hemophilia B and other bleeding disorders using FIX-Padua in AAV gene therapy
- Additional patents are pending in Europe
- Professor Simioni from the University of Padua is the inventor for this patent family
  - Acting as an advisor and consultant for uniQure

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AMT-130: Huntington's disease

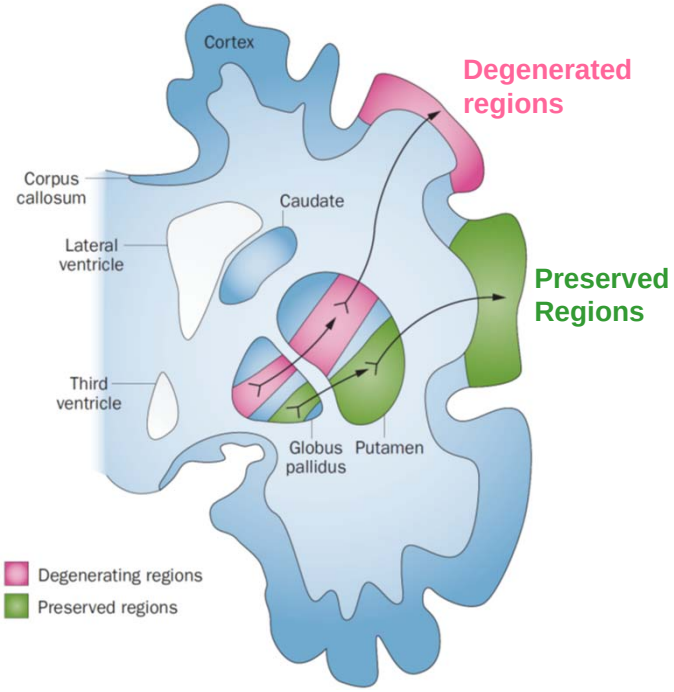
Huntington's disease

- Caused by expansion of CAG trinucleotide in exon 1
- No disease-modifying therapies available
- Prevalence of ~60,000 to 70,000 patients in US and EU
  - Significant additional patients undiagnosed

Therapeutic Targeting

- mHTT leads to damage in the striatum, and in later stage disease, in parts of the cortex
- Therapeutic targeting of ***striatum (caudate nucleus and putamen), globus pallidus and cortex (sensorimotor)***

Huntington's Disease Pathway



Ross CA., et al. Nat Rev Neurol. 2014 Apr;10(4):204-16

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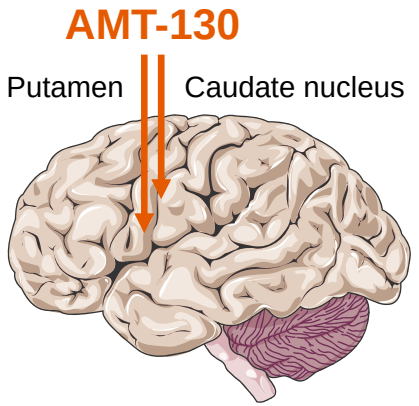
AMT-130: treating Huntington's disease

AMT-130 gene therapy for Huntington's disease (HD)



- Non-selective **knockdown of huntingtin protein** (HTT) in the brain
- Utilizes **proprietary miRNA** that binds to and degrades HTT mRNA
- **One-time injection** in the striatum, the primary affected structure in HD
- AAV5 has been shown to have **widespread distribution in brain, including cortex**
- MRI-guided stereotactic administration directly into **deep structures of brain**
- **Demonstrated preclinical PoC** in multiple small and large animal models
- AMT-130 **leverages same manufacturing platform** and process used for AMT-061

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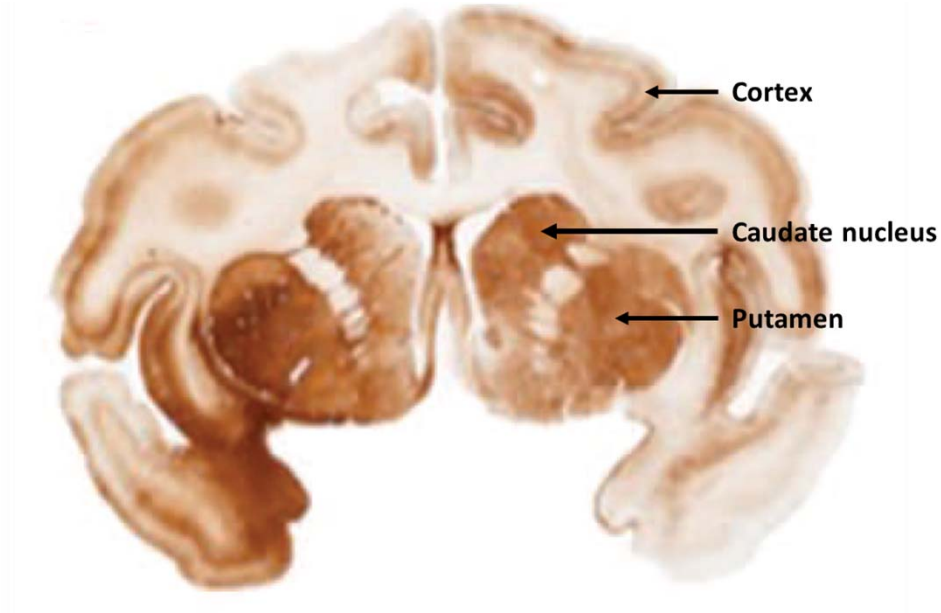
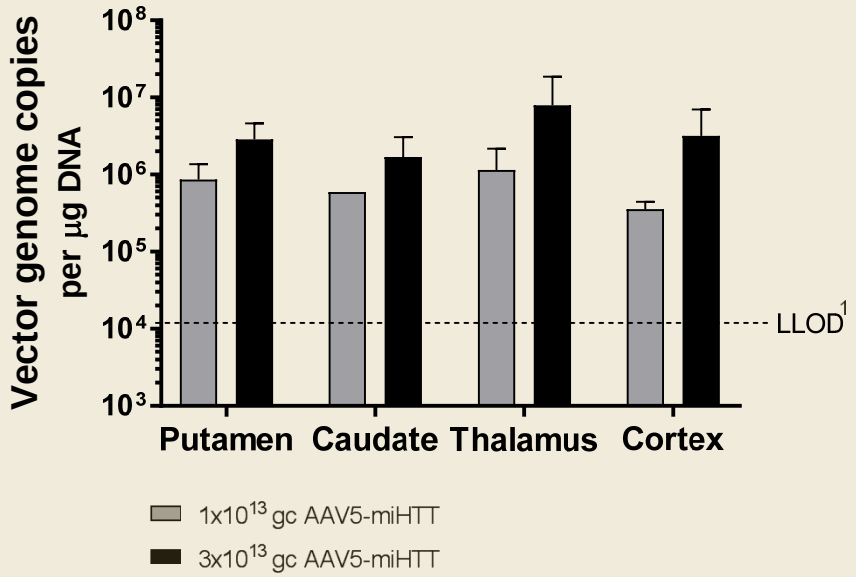
AMT-130: widespread distribution in brain

Penetration throughout NHP brain



Samaranch L. et al, Gene Ther. 2017 Apr;24(4):253-261. Figure 3

Vector DNA distribution



[1] Lower Limit of Detection

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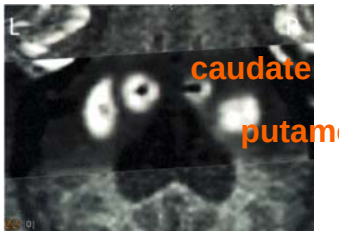
AMT-130: strong reduction of mHTT

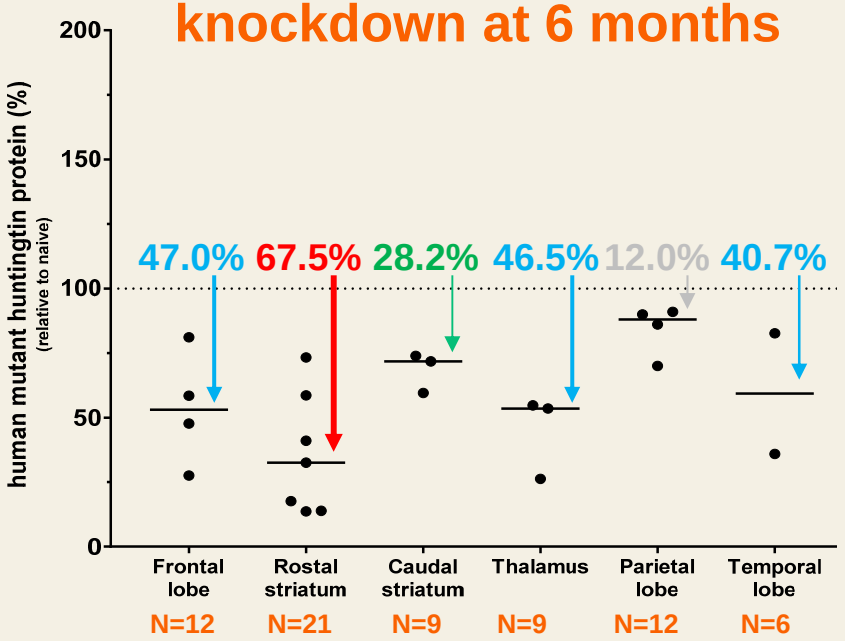
Libechov transgenic (tgHD) minipigs:

- Life-span: 12-20 years
- Body weight: 50-140 kg
- Brain weight: 90-100 g
- Highly developed immune system

MRI-guided CED



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
- Completing safety toxicology studies
- Granted Orphan Drug Designation by FDA
- Granted Orphan Medicinal Drug Designation by EMA

Next Steps

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

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