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 future development of our programs in Sanfilippo B, Hemophilia B and cardiovascular diseases, the success of our collaboration with Bristol-Myers Squibb, 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 2014 Annual Report on Form 20-F filed with the Securities and Exchange Commission on April 7, 2015.

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.
uniQure – Leading the Field in Gene Therapy

Three therapeutic franchises with established clinical PoC in two indications
- Liver / Metabolism
- CNS Disorders
- Cardiovascular Disease (Partnered w/ BMS)

Deep clinical pipeline enabled by validated technology
- Completed Ph I/II in Sanfilippo B
- Ongoing Ph I/II in Hemophilia B
- Ongoing Ph I/II in Parkinson’s disease

Commercial-grade manufacturing in place in two geographical locations
- Stable, consistent, high-quality, validated, scalable

Proven, proprietary AAV vector platform supports de-risked expansion of pipeline
- Safe and effective delivery into multiple tissues, wide range of therapeutic opportunities
AAV5 - uniQure’s Proprietary and Proven Vector

- Validated technology
- 23 patients successfully treated
- Lowest prevalence of pre-existing antibodies
- Successful delivery in liver and brain tissues
- Broad applicability across multiple therapeutic areas
- Proven safety in three clinical trials
Leadership in Evolution of Gene Therapy Technology

Improved delivery (vector), higher expression efficiency and specificity (promoters)

• uniQure will continue to invest significantly in its technology platform to enhance the functionality and applicability of gene therapy to patients
  – Improved vector and promoter technology to encapsulate larger genes, increase expression efficiency
  – Advanced promoters to increase penetration into target tissues
  – Regulated gene expression (up and down)
  – Re-administration protocols

• Goal is to build the premier gene therapy company, ensuring growth of pipeline
uniQure’s Leading Baculovirus Manufacturing Platform

Validated, superior quality, scalable and cost-efficient

Features
- EU-approved in Amsterdam
- One of the largest, most versatile manufacturing worldwide in Lexington
- Scalable to 2 x 2000L
- Cost effective
- Ready for commercial scale-up

Benefits
- Control process all the way through commercialization
- Lower risk scale up
- Cost effective and scalable
- Adaptable to every project
- High volume capacity
- Consistent, high-quality
Pipeline Overview - Reproducible Platform Enables Deep Pipeline Across Three Therapeutic Areas

Liver / Metabolism
- Hemophilia B
- Hemophilia A
- Multiple research programs

CNS
- Sanfilippo B
- Parkinson’s disease (GDNF)
- Huntington’s disease (miRNA)
- Multiple research programs

Cardiovascular
- CHF (S100A1)
- 3 undisclosed targets

Preclinical | Phase I/II | Phase III
--- | --- | ---
Chiesi (EU+ only) | uniQure | uniQure

Partners and Academic
- uniQure
- Partnered
- Academic
Liver / Metabolism Gene Therapy Portfolio

Success with AAV5 in Hemophilia B Leads to a Growing Pipeline of Gene Therapy Approaches in the Liver
Preliminary Top-line Results Validate AAV5 Vector

Phase I/II AMT-060-01 study – Low-dose Cohort

**Trial Design**
- 10 patients
- 2 cohorts
- 9 sites

**Inclusion Criteria**
- Older than 18 Years
- Severe (<1% FIX) or moderate-severe (FIX<2%) levels
- On prophylactic or on-demand FIX
- Severe bleeding (>4 bleeds/year or arthropathy)
- >150 exposure days to FIX

**Top-Line Results**
- First two patients achieved FIX expression levels of 5.5% and 4.5% of normal (wk 20 and 12, respectively)
- **Four of the five patients fully discontinued prophylactic rFIX** as of January 6, 2016
- One patient experienced a mild, transient and asymptomatic elevation of transaminase levels
- No elevated transaminase levels have been observed in other patients
Transforming Severe Hemophilia B into a Mild Disease

Severe to Moderate Phenotype

Prophylactic Treatment: 1 – 3 infusions/wk

- Frequent/recurring spontaneous bleeds
- rFIX prophylaxis required 1-3 times/week
- Long-term impairment in mobility
- Severely affected quality of life
Transforming Severe Hemophilia B into a Mild Disease

**Severe to Moderate Phenotype**

Prophylactic Treatment: 1 – 3 infusions/wk

- Frequent/recurring spontaneous bleeds
- rFIX prophylaxis required 1-3 times/week
- Long-term impairment in mobility
- Severely affected quality of life

**Mild Phenotype**

Low Risk for Spontaneous Bleeds; No Prophylaxis

- One-time administration
- Bleeding only in response to surgery or injury
- Higher quality of life; No functional impairment
- Significant reduction in cost of care
## Validated Gene Cassette

**Demonstrated long-term durability**

<table>
<thead>
<tr>
<th></th>
<th>St. Jude Study (data with <strong>highest</strong> dose, AAV8)</th>
<th>AMT-060-01 Study (data with <strong>starting</strong> dose, AAV5)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Factor IX Expression</td>
<td>Therapeutically relevant</td>
<td>Positive preliminary data</td>
</tr>
<tr>
<td>Shift in severity of phenotype</td>
<td>Yes</td>
<td>Positive preliminary data</td>
</tr>
<tr>
<td>Reduction in use of replacement therapy</td>
<td>96%</td>
<td>4 out of 5 patients off prophylaxis</td>
</tr>
<tr>
<td>Reduction in bleeding</td>
<td>94%</td>
<td>Same gene cassette</td>
</tr>
<tr>
<td>Long term safety</td>
<td>Favorable</td>
<td>Potentially more favorable</td>
</tr>
<tr>
<td>Life changing therapy</td>
<td>Yes</td>
<td>Same gene cassette</td>
</tr>
<tr>
<td>Durability</td>
<td>5+ years</td>
<td>Same gene cassette</td>
</tr>
</tbody>
</table>
AMT-060 in Rhesus Monkeys

- AMT-060 mediates hFIX expression in Rhesus monkeys at similar levels to published data (Nathwani et al. 2007)
  - $5 \times 10^{12} \text{ gc/kg} \rightarrow \sim 5\% \text{ FIX expression}$
- Dose-dependent expression was confirmed in Cynomolgus monkeys (GLP study)

AMT-060 in Cynomolgus Monkeys

- hFIX increases with dose

Expected for High-dose Cohort: Preclinical Basis for AMT-060 Dose-mediated Expression
Phase I/II AMT-060-01 Study – Next Steps

1. Completed enrollment of high-dose cohort
2. Low-dose cohort data presentation at EHA
3. DMC data review before initiating high-dose cohort
4. Completed enrollment of high-dose cohort
5. Establish regulatory pathway for approval
6. High-dose cohort data presentation before end of 2016
CNS Gene Therapy Portfolio

Success with AAV5 in the CNS paves the way for pipeline expansion with several disease modifying approaches.
Success with Lead Indication Supports Expansion

Significant number of rare and orphan CNS-based diseases with no current treatment options well-suited to a gene therapy approach

- Intracranial administration protocols established
- Modular approach supports rapid development

**Monogenetic autosomal recessive diseases with missing protein**

**Sanfilippo B syndrome:**
> 50 additional lysosomal storage diseases caused by missing enzyme

**Monogenetic autosomal dominant toxic gain-of-function**

**Huntington’s disease:**
knock-down of toxic allele

**Restoration of function through neurotropic support**

**GNDF in Parkinson’s disease,**
potentially other neurodegenerative diseases with large commercial indications
AMT-110 Data at 12-month Follow-up

Encouraging efficacy data

Robust Biomarker Results

- All 4 patients achieved active NaGlu enzyme levels of 14-17%, baseline had been 0% at time of dosing
- Presence in CSF indicates effective transmission, level of protein production in brain may be higher

Encouraging Signs of Efficacy

- All 4 patients continued to gain skills, measured with 3 different cognitive tests
- Youngest patient gained most skills, supporting information for pivotal trial design

MRI data suggests no progression of brain atrophy
Transfer of sponsorship of Ph I/II extension protocol to uniQure

30-month follow-up data from all 4 patients in early 2017

Consolidate available SFB natural history data

Establish regulatory pathway to approval
Huntington's Disease Program Overview

Target indication - Reduction of mutant aggregating huntingtin to decrease toxic burden

- Worldwide prevalence of 2.71 in 100,000\(^1\);
- EU/US 5.70 in 100,000\(^1\)
- No treatment available

Data to Date
- Lead selection completed

Status
- Non-clinical safety toxicology studies ongoing

Next Steps
- Peer review publication of preclinical data
- Initiate first-in-man study

\(^1\)Pringsheim et al. Mov. Disord. (2012)
Cardiovascular Program

The uniQure Bristol-Myers Squibb Collaboration – Changing the Landscape of Cardiovascular Medicine
uniQure & Bristol-Myers Squibb Collaboration

Largest gene therapy deal leveraging uniQure’s technology platform

- Up to 10-target collaborations providing exclusive rights to BMS in CV disease
- Exclusive license for S100A1 gene therapy program for chronic heart failure
- Leverage BMS clinical and commercial expertise in CV disease
- $140 million received to date and $2.3 billion in potential milestones
- Up to double-digit royalties
- All R&D paid by BMS
- QURE exclusive manufacturer
- BMS has 9.9% stake in uniQure
- Warrants to own up to 19.9%

S100A1 Gene Therapy Heart Failure Program

Acquired CV Diseases

Non-acquired CV Diseases
uniQure – Leading the Field in Gene Therapy

Deepest pipeline in gene therapy
Three ongoing clinical programs and multiple preclinical candidates across three key therapeutic areas

Proven AAV vector and technology platform
Proof of concept in the liver and brain

Industry-leading manufacturing capabilities
Ability to scale from early development to commercial scale

Landmark collaboration with BMS in cardiovascular disease
10 target collaboration worth up to $2B

Strong financial position
$230 million (€203.5 million) in cash as of Dec 31, 2015
Thank You!

From the uniQure Team