

uniQure

**A Dutch success comes to life through effective
partnering strategies**

Dutch Life Sciences conference
November 2015

Most Advanced Gene Therapy Company

Deep Portfolio and Validated Technology Platform

**Leading fully
integrated
AAV-based
gene therapy
company**

Near term clinical results in Hemophilia B, Sanfilippo B (MPSIII)

> \$2.3 bn BMS partnership for cardiovascular gene therapies

Glybera first EU-approved gene therapy, via partner Chiesi

Deep product pipeline targeting liver, CNS, CV diseases

Industrial scale, insect cell-based manufacturing platform

Strong next-generation vector + promoter partnerships

Ample capital to execute our business plan

Proprietary Manufacturing and Vector Know-How

The Core of Our Technology Platform

LEADING MANUFACTURING CAPABILITIES

> AMSTERDAM, NL (EU)

- > 2 x 50 L
- > EMA approved facility

> LEXINGTON, MA (USA)

- > World's largest GT manuf facility
- > 2 x 500 L (scalable to 2 x 2,000 L)
- > GMP validation in 2H 2015



> PROPRIETARY INSECT CELL-BASED TECHNOLOGY

- > Enhanced proprietary replication process; Safe by design
- > Highly scalable and cost efficient
- > Attracting collaborations and partnerships

STATE-OF-THE-ART VECTOR DESIGN

Natural AAV Serotypes

Directed Evolution

Next-Gen AAV

AAV1 (exclusive for LPLD/ muscle)

AAV2 (public domain)

AAV5 (exclusive for Liver/ CNS)
(non-exclusive for CV)

Super-mutant vectors

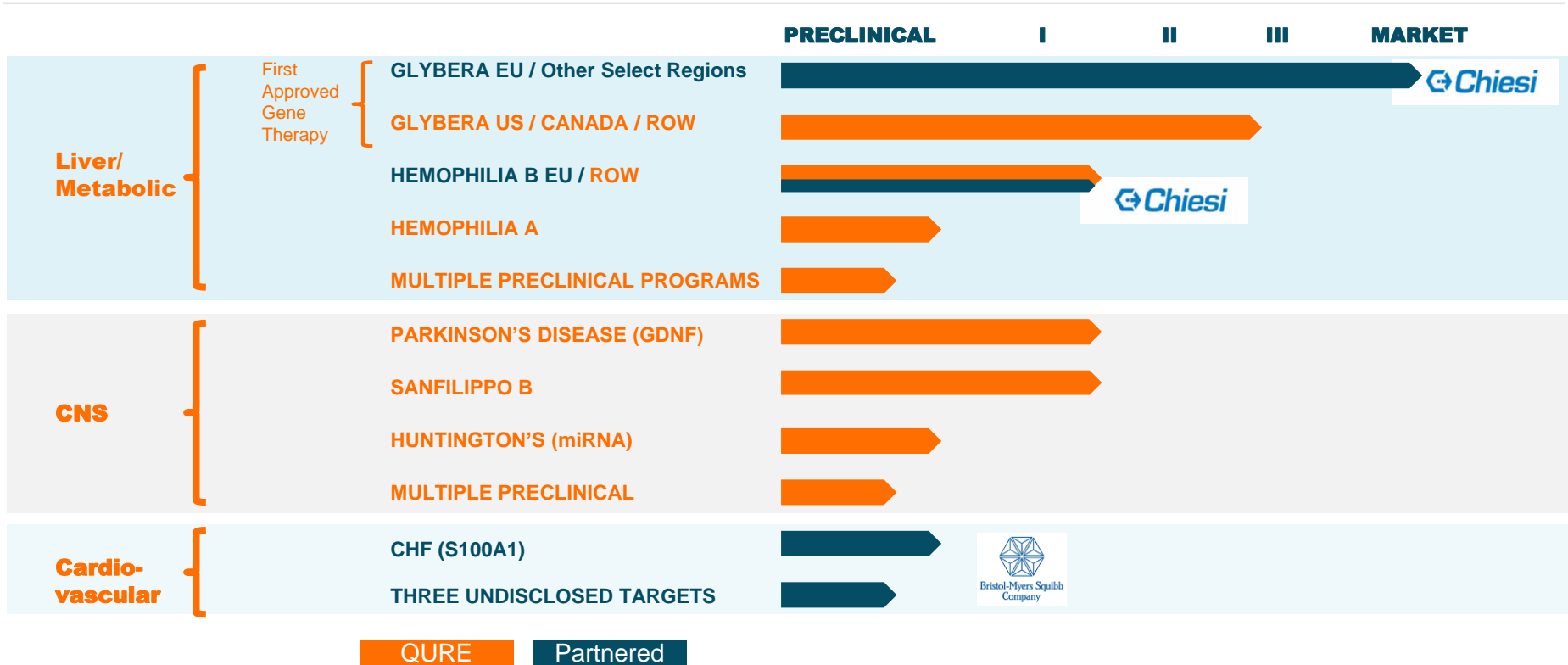
Selected for potency, IP and FTO

Available by end of 2015



Pipeline Overview

Establishing a Deep Pipeline Across Three Therapeutic Focus Areas



Gene Therapy Industry Ready For Prime Time

Recent Successes and Failures



- + RPE65 PH 3 DATA
- + SANFILIPPO B PH 1/2 – 1ST LSD POC
- + GLYBERA LAUNCH/ PRICING
- + POC SICKLE CELL DISEASE/ BETA THALASSEMIA
- + MULTIPLE PRECLINICAL SUCCESSES (INCL. GLYBERA APPROVAL BY EMA + S100A1)
- + HEMOPHILIA B – DURABLE EFFECT IN HUMAN LIVER
- + JEAN ERICKSON 1ST CLINICAL BENEFIT IN PARKINSON'S
- + RESTORED IMMUNE SYSTEMS IN 17 SCID PATIENTS STARTING IN 2004

2015

- AVALANCHE - MACULAR DEGENERATION
- CELLADON - HEART FAILURE
- CEREGENE – PARKINSON'S
- SEVERAL HEMOPHILIA TRIALS
- TARGETED GENETICS- CF PHASE 2
- FAILED HEMOPHILIA FIX TRIAL
- FAILED X-SCID TRIAL IN 2002



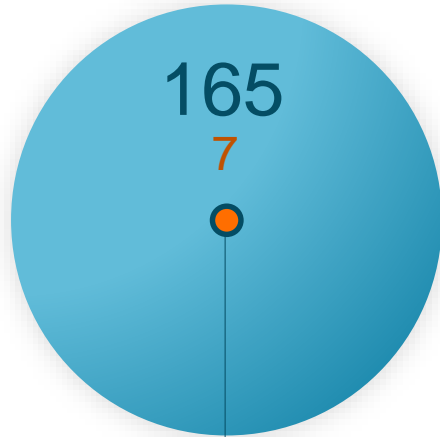
2005

Before

Gene Therapy – Exponential Growth

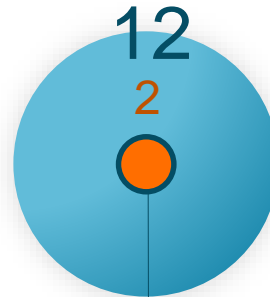
Clinical Trial #s and Market Cap of Listed Companies Exploded Over Past 10 Years

● 2005 ● 2015



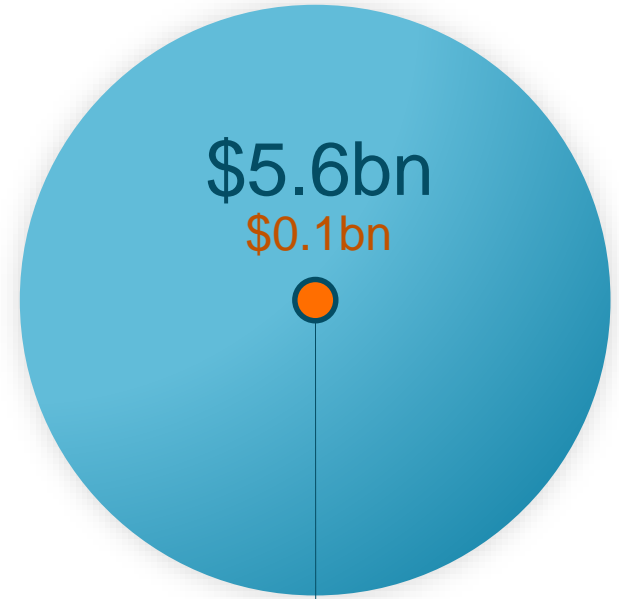
OPEN CLINICAL TRIALS

(clinicaltrials.gov)



PUBLIC COMPANIES ¹⁾

1) 2005: TGEN, OXB; 2015: QURE,AAVL, BLUE, ONCE, AGTC, ARWR, CLDN, RGNX, SGMO, ABEQ, DMTX, VYGR



MARKET CAP PUBLIC CO'S

(as of 10/12/2015)

uniQure in 2013

2013

EMPLOYEES



45

PARTNERS

> ACADEMIC COLLABORATIONS

LOCATIONS



> AMSTERDAM,
NL

CLINICAL PROGRAMS



(EU-approved)

CASH



US\$ 0.4m

MARKET CAPITALIZATION



< US\$ 50m

... has also grown exponentially

2015

EMPLOYEES



PARTNERS

- > ACADEMIC COLLABORATIONS
- > BMS, CHIESI
- > 4D THERAPEUTICS (NEXT GEN VECTORS)
- > SYNPROMICS (NEXT GEN PROMOTORS)
- > TREEWAY FOR ALS

LOCATIONS

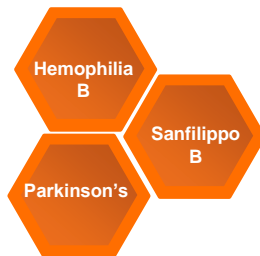


- > AMSTERDAM, NL
- > LEXINGTON, MASS. US
- > HEIDELBERG, GERMANY

CLINICAL PROGRAMS



(Commercial)



CASH



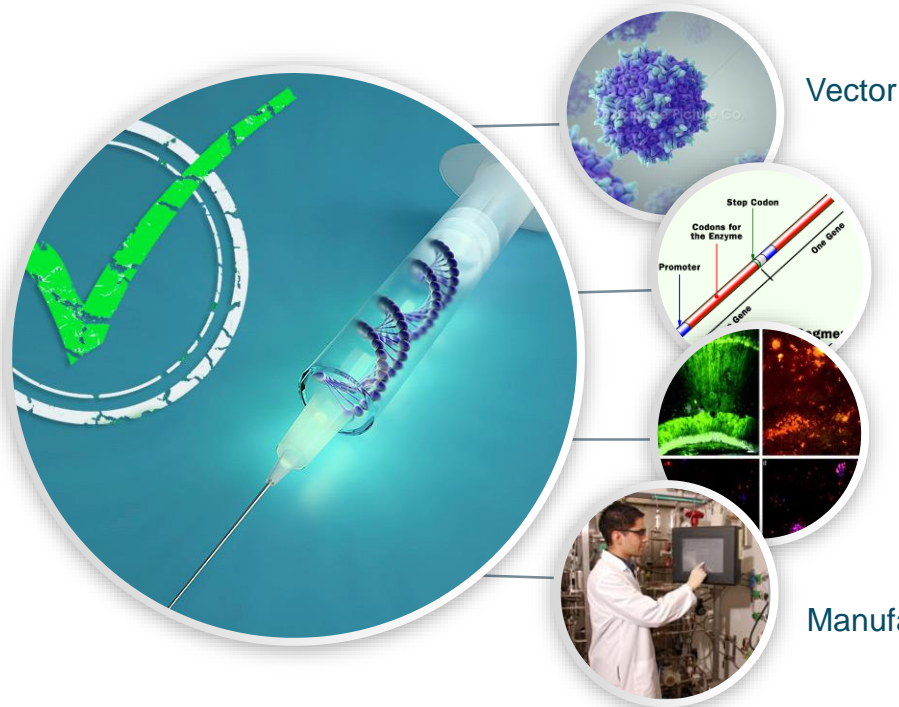
MARKET CAPITALIZATION



US\$ 400m

uniQure – Past Years Focus

Vector, Promoter, Administration, Manufacturing Technologies Continued to Evolve



Vector

POTENCY

- > Proprietary AAV5
- > Rational design - in house next gen optimization
- > Directed evolution w/ 4D Therapeutics

Promoter

SPECIFICITY

- > Solve “Efficiency/ Size” dilemma
- > Partnersip w/ Synpromics

Administration

FIT FOR PURPOSE

- > In-house development for CVD
- > CNS administration with UCSF

Manufacturing

SAFETY, SCALE, QUALITY

- > Largest industrial scale manufacturing
- > Safe-by-design

uniQure – The Next 5 Years

Delivering on the promise of gene therapy

Changing
the World of
Medicine with
Gene-based
Therapies

GLYBERA LAUNCHED, SANFILIPPO B PH 1/2 SUCCESSFUL,
HEMOPHILIA B PH 1/2 EXPECTED EOY

BECOME A FULLY INTEGRATED GENE THERAPY
COMPANY WITH UP TO 2 MARKETED DRUGS* IN 2020

THE LEADING GENE THERAPY CLINICAL PORTFOLIO ...

... PROPRIETARY PROGRAMS FOCUSED ON
RARE AND OTHER GENETIC DISEASE

... PARTNERING PROGRAMS IN LARGER INDICATIONS

*) beyond Glybera

Patients First!

From Technology Platform to Therapeutic Areas

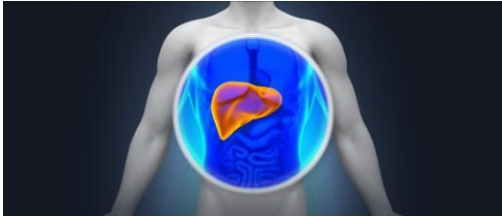
uniQure Technology – Basis of Success

Vectors, Promoters, Manufacturing, Development, Regulatory



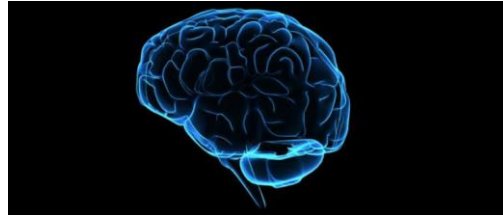
LIVER/METABOLIC

Therapeutic Focus Area



CNS

Therapeutic Focus Area



CARDIOVASCULAR

Therapeutic Focus Area



uniQure Strategy



PATIENTS FIRST!

**BUILDING A
PORTFOLIO OF
PRODUCTS ACROSS 3
THERAPEUTIC FOCUS
AREAS**



FAST TO MARKET

**LEVERAGE OUR
REGULATORY AND
CLINICAL
EXPERTISE**



PARTNERSHIPS

**FOR LARGER
PATIENT
POPULATIONS
(E.G. BMS)**



TECHNOLOGY LEADERSHIP

**CONTINUE TO
DEVELOP NEXT
GENERATION
TECHNOLOGIES**



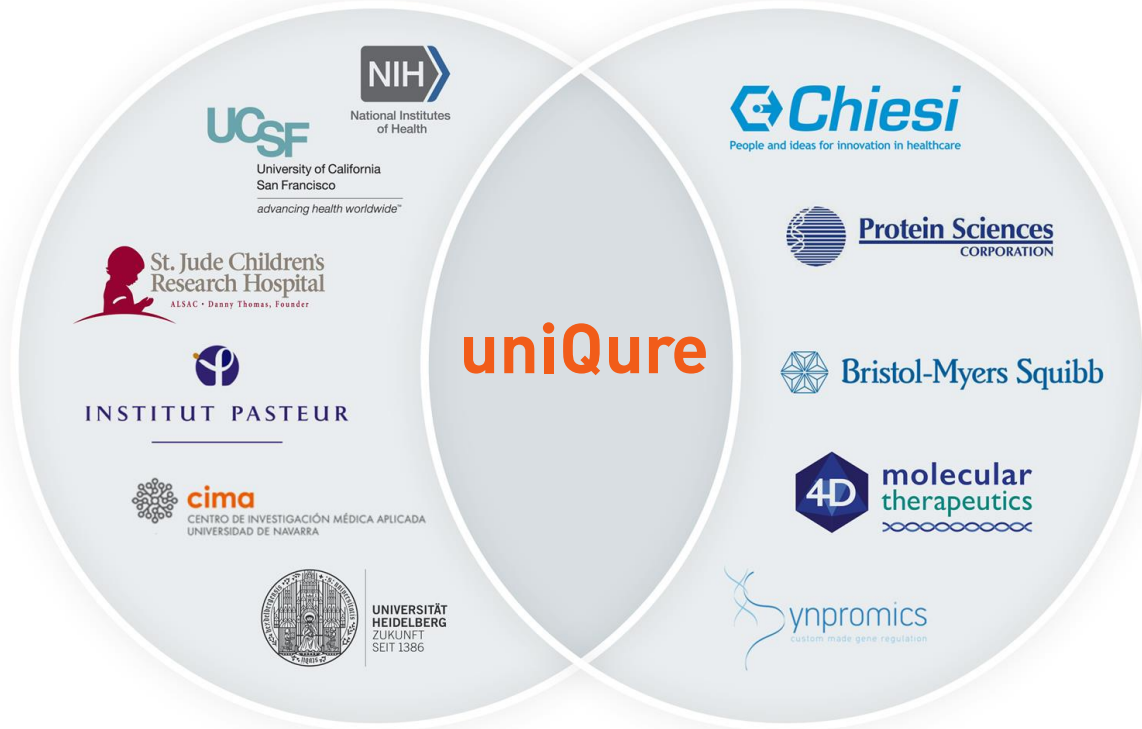
INTERNAL AND EXTERNAL GROWTH

Benefiting From The World Around Us

Academic and industry partnerships to accelerate growth



ACADEMIA



INDUSTRY

2015 A Breakthrough Year For uniQure

Delivering the promise of gene therapy to patients suffering from rare and other debilitating diseases.



**CLINICAL
DATA FROM**

**SANFILIPPO B
HUMAN TRIAL**



**MULTI-BN \$
PARTNERSHIP
WITH BMS**

**DESIGNATION
OF 3 ADDITIONAL
COLLABORATION
TARGETS**



**1ST GENE
THERAPY
LAUNCH**

**IN EUROPE
GLYBERA**

Q



**3 THERAPEUTIC
FOCUS AREAS**

**A GREAT
EXPERIENCED
LEADERSHIP
TEAM**



**+\$220
MILLION**

**SECURED
YTD**



**HUMAN POC
TOP LINE DATA**

**HEMOPHILIA B
1 EXPECTED
END OF YEAR**

uniQure