ebe european biopharmaceutical enterprises + efpia European Federation of Pharmaceutical Industries and Associations

INCENTIVES, IP AND SMALLER COMPANIES

Case study:

uniQure

Who

uniQure, based in Amsterdam in the Netherlands and Lexington, Massachusetts, USA, is developing curative gene therapies for patients with severe genetic diseases. The company had around 220 employees in 2017.

Contribution

- **Ongoing clinical program for Hemophilia B,** a severe orphan blood clotting disorder, causing internal or external bleeding. uniQure gene therapy product candidate AMT-061 could restore blood-clotting with one single or very few administrations, preventing frequent intravenous Factor IX injections.
- **Preclinical proof of concept for a gene therapy for Huntington's disease,** a rare fatal neurodegenerative genetic disorder. No therapies available to delay onset or slow progression to total physical and mental deterioration. uniQure's product candidate AMT-130 may turn off production of the disease-causing huntingtin protein with direct delivery of artificial micro-RNA to the brain.

Role of incentives

US Breakthrough Therapy designation & EMA PRIME status

- prospect of accelerated assessment for marketing authorisation

Orphan designation (OD) in Europe

- ten years of market exclusivity for approved products
- focused conversations with health authorities and scientific advice at no cost

Advanced Therapy Medicinal Product (ATMP) status

- centralised marketing authorisation procedure in the EU
- pooled expertise and direct access to the entire EU market after registration

Combination of OD & ATMP & SME designation

- up to 100% reductions in fees for administrative services, Scientific Advice procedures, Marketing Authorisation Application (MAA) and regulatory pre-inspections
- > 80% reductions in fees for post-authorisation and pharmacovigilance procedures

MedDRA licence-fee waiver

- free electronic pharmacovigilance reporting pre- and post-authorisation

Paediatric investigational plan (PIP)

- children and juvenile patients benefit as early as possible
- market exclusivity 10 + 2 years with OD & PIP at marketing authorization

History

2012 • first-ever marketing authorisation for familial lipoprotein lipase deficiency (LPLD)

2016 • preclinical proof of concept for Huntington's disease gene therapy

2017 • Hemophilia B: US-FDA breakthrough and EMA PRIME designations
• Huntington's disease: advancing clinical trials; ODD designation by US-FDA

What's next

- Pivotal Phase III in severe and moderately severe hemophilia B
- New gene therapy to treat hemophilia A in pre-clinical evaluation
- Huntington's disease: IND application and clinical development for AMT-130

For the whole story of uniQure see:
https://www.ebe-biopharma.eu/media-centre/publications and
https://efpia.eu/news-events/the-efpia-view