

How to make exploitable research?

9th European Conference on Rare Diseases & Orphan Products
Vienna, 10-12 May 2018

Session 0102: Research: from an idea to the real world
11 May 2018 16:30-18:00

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FONDAZIONE



About Fondazione Telethon

FONDAZIONE



- Italian biomedical charity focused on **genetic diseases**
- **Founded in 1990** at the behest of a group of **patients**
- Supported through **fundraising**

Our mission

To advance biomedical research towards the cure of muscular dystrophy and other genetic diseases

Our vision

To **convert** the **results** of excellent, selected and sustained research **into available therapies**

We give **priority to rare genetic diseases** that are **neglected** by major public and private investment

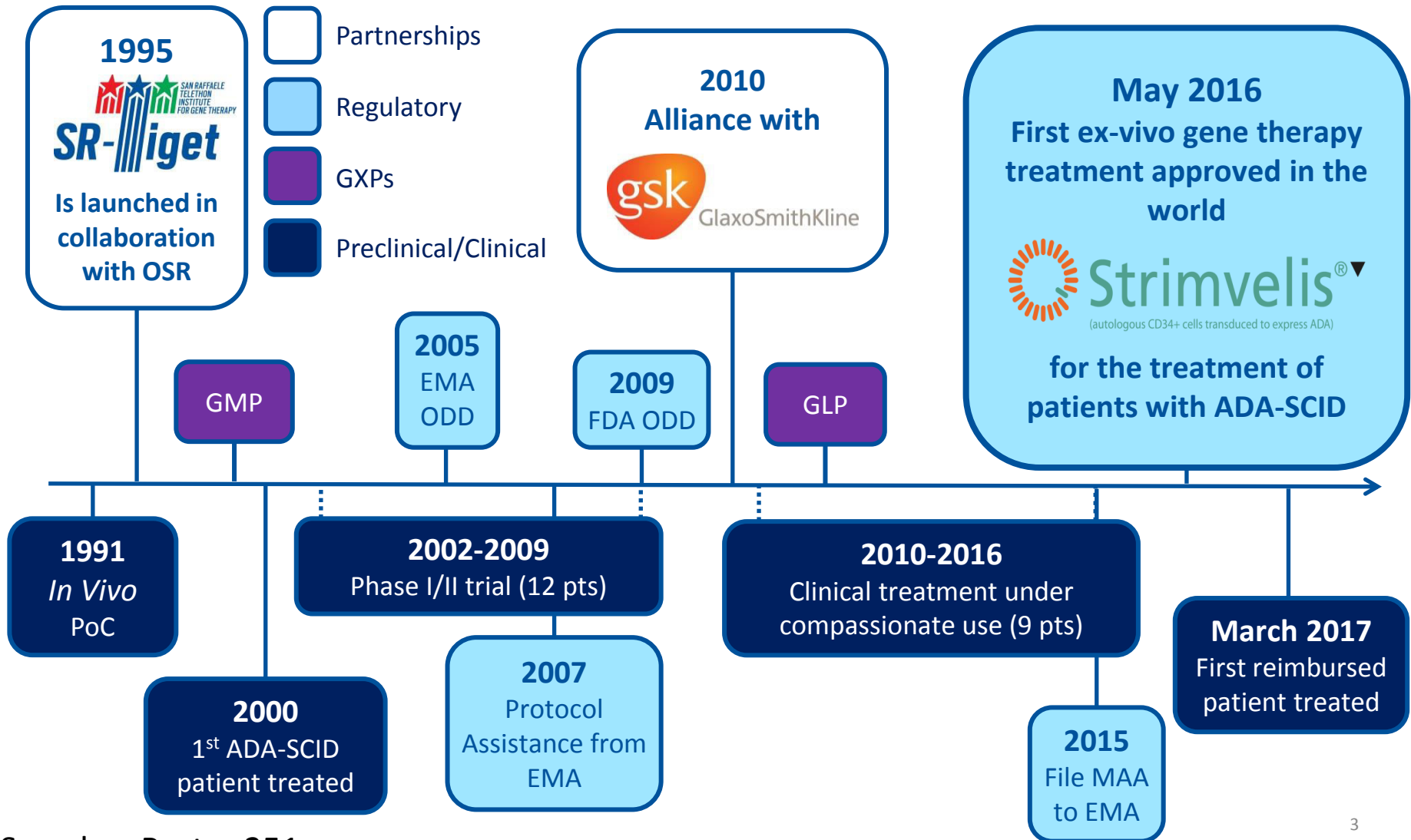
Excellence-driven grant allocation

KEY FIGURES 1990-2017

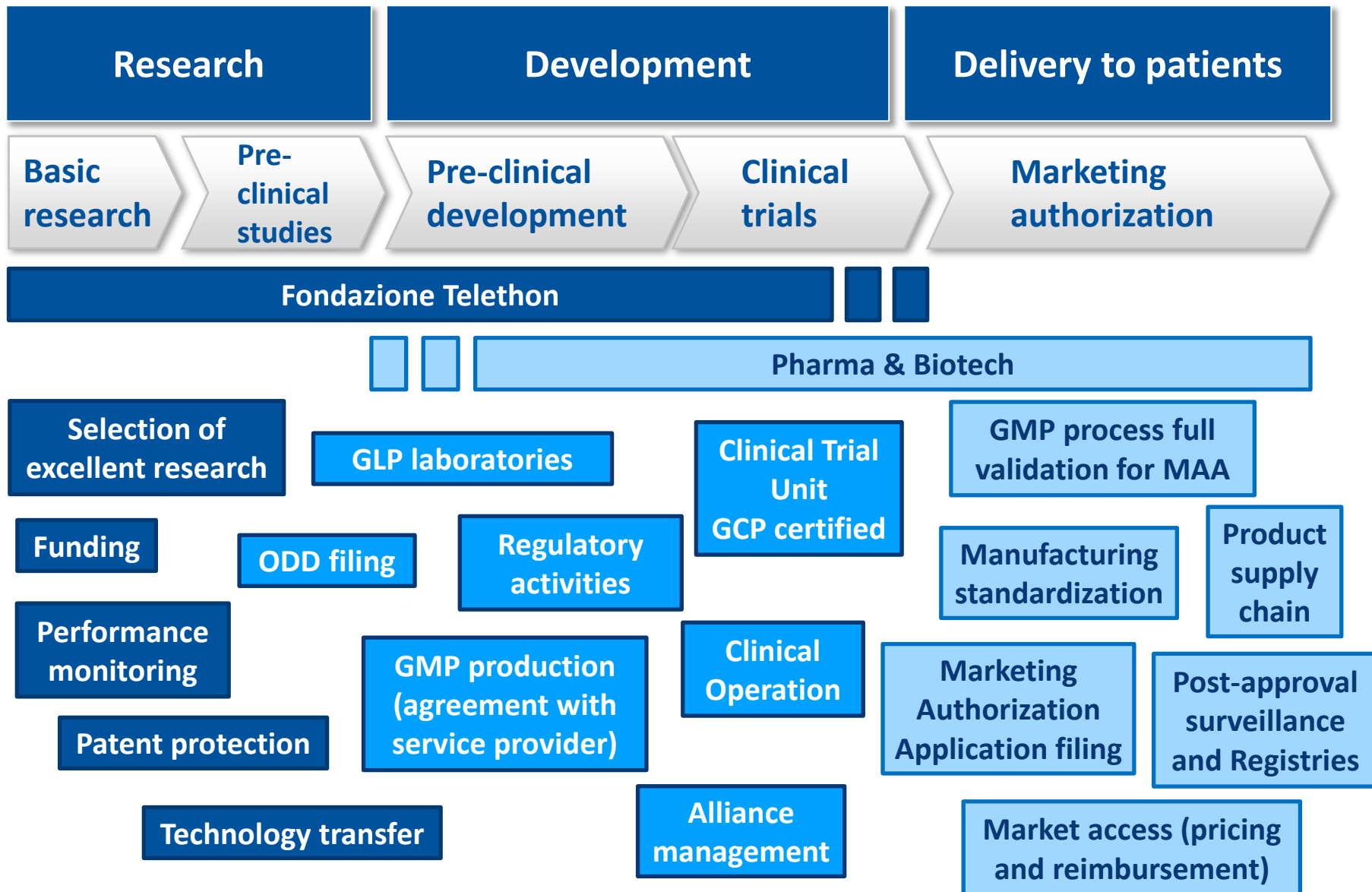
- 498 M€ research investment
 - 2,629 research grants
 - 1,611 PIs awarded
 - 571 genetic diseases studied
- **Strategic initiatives** (intramural research)
 - **Competitive grants** (funding programs in Italy)

Research development up to available therapies

A successful case history: Strimvelis®



The research development pipeline: a collaborative model



The pillars of Fondazione Telethon's industrial agreements

The agreements between Fondazione Telethon and industrial Partners

- Safeguard **research independence** of Telethon investigators
- Retain **intellectual property rights**

- Mandate **commitment in developing therapies**
- Imply **return of any IP** and **results co-developed**, in case the Partner does not pursue therapy development up to patient access

- Provide **funding** in support of the research in the collaboration programs
- Supply **additional funding** through milestones/royalties, in support of further/other research activities

Focus on Orphan Drug Designation (ODD)

- Eligibility prerequisites for grant applications (e.g. H2020)
- Pre-submission meeting available
- Reduced fees for protocol assistance after ODD
- Market exclusivity
 - EMA has a dedicated section for academia on its website:
http://www.ema.europa.eu/ema/index.jsp?curl=pages/audience/alp_audientype_000006.jsp&mid=
 - EU and USA apply different rules and requirements for both ODD and scientific advice

Hints

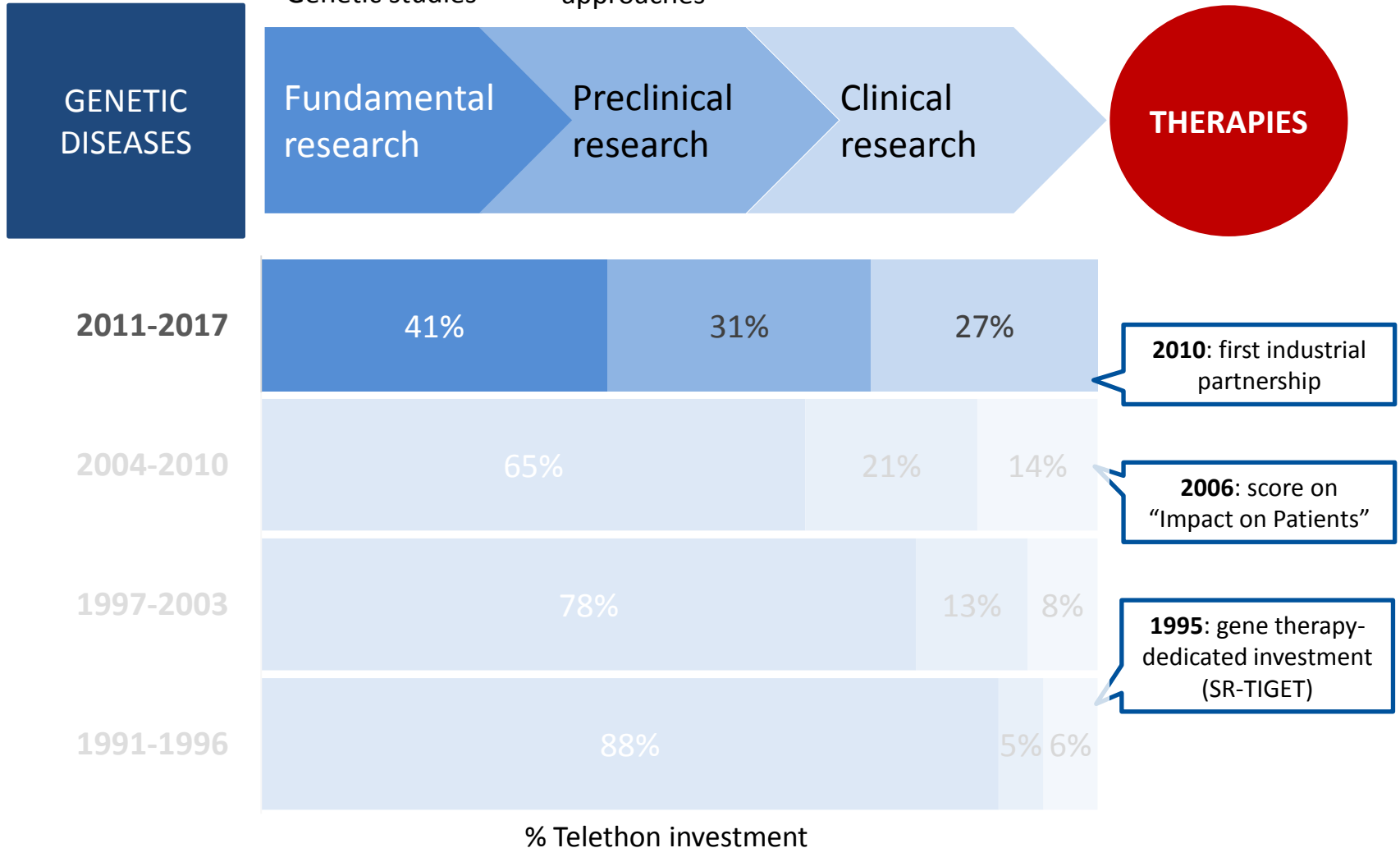
- This procedure can be submitted anytime during the product development, however you need to have robust functional data
- Plan well in advance as the procedure has fixed timelines
- Comply with EU and national GLP requirements in conducting preclinical studies
- Secure the right resources to work on this procedure
- No budget needed for ODD

Fondazione Telethon's research portfolio



Driving research towards therapies

- Disease mechanisms
- Genetic studies
- In vivo/in vitro therapeutic approaches
- Therapeutic trials
- Observational studies



Monitoring for translation: Fondazione Telethon's competitive grants

Monitoring procedure

- Validation of the project's objectives (proposal): disease, approach, models
- Monitoring progress (published results, progress reports)
- Direct enquiry with PI (meeting/teleconference)

Assessment Criteria

- Trial readiness (natural history, clinical network, access to patients, etc.)
- Access to CRO for pre-clinical studies
- Regulatory steps (ODD, protocol assistance)
- Clinical competence
- Competitiveness
- Exclusivity (IP ownership)
- Technology transfer competence at HI
- Access to GMP facilities



Pilot activity on Telethon grants awarded in 2016-2017

- Starting pool: 37 projects on preclinical or clinical studies
- 8 projects identified as mature for translational development

Sofinnova-Telethon venture capital fund

- Launched by Sofinnova Partners (February 2018)
- Fondazione Telethon participates as advisor

A new way forward to promote the creation of Italian biotech startups focused on rare genetic diseases

Target: investment in 15-20 start-ups

- 40 Million Euros committed by European Investment Fund and Cassa Depositi e Prestiti
- 10 million Euros committed by the European Investment Bank
- 25-50 Million Euros being raised from private investors

→ Fondazione Telethon will propose projects to be developed into new therapies

→ The fund will be managed by Sofinnova

Enabling factors for competitive/transformational research

- **Excellent fundamental and pre-clinical research**

- Stringent selection system (scientific merit, impact on patients)
- Adequate funding
- Monitoring research progression and results



- **Identifying projects with translational potential**



- **Effective translational research**

- Intellectual property protection and technology transfer
- Compliance with “GXP” requirements
- Management of regulatory affairs
- Management of clinical trials
- Competences in drug development

Thank you!

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