BOUSSIAS

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EU Cross-border healthcare and its application to Rare Diseases

FONDAZIONE Telethon

Gabaldo Michela
Head Alliance Management & Regulatory Affairs
Fondazione Telethon

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Relevance of Cross-Border Access to Rare Disease

- **Complex** or **rare** diseases and conditions require **highly specialised treatment**
- **Ultra rare** diseases affect only a **few patients per year in Europe**
- **Concentration of knowledge and resources**, not available in all countries

Relevance of Cross-Border Access to *Orphan ATMPs*

- **ATMPs** may need to be administered by **trained/certified healthcare providers** or in highly **specialised centers**, not necessarily available in all countries.

- Many ATMPs are autologous, requiring **specific logistic requirements** and may be better addressed if administered only in a limited number of specialised centres.

STRIMVELIS® case study

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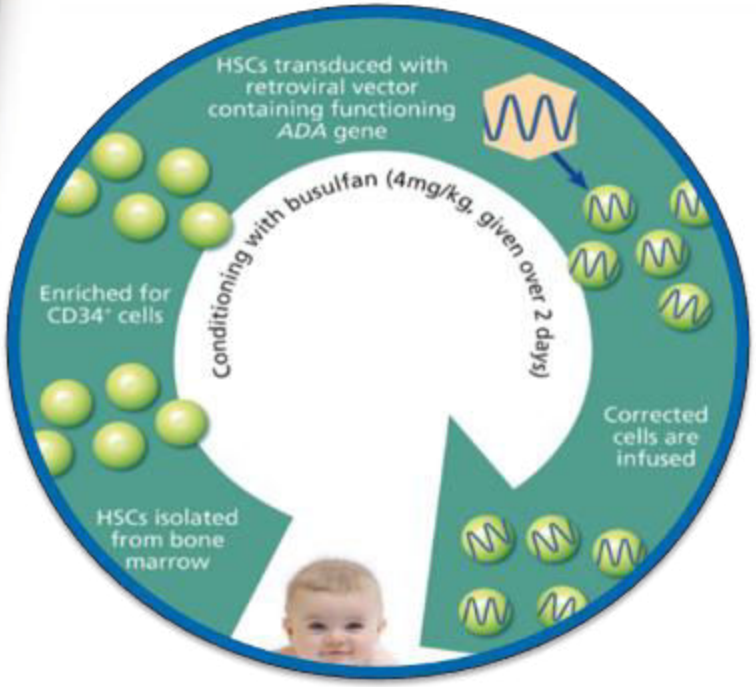
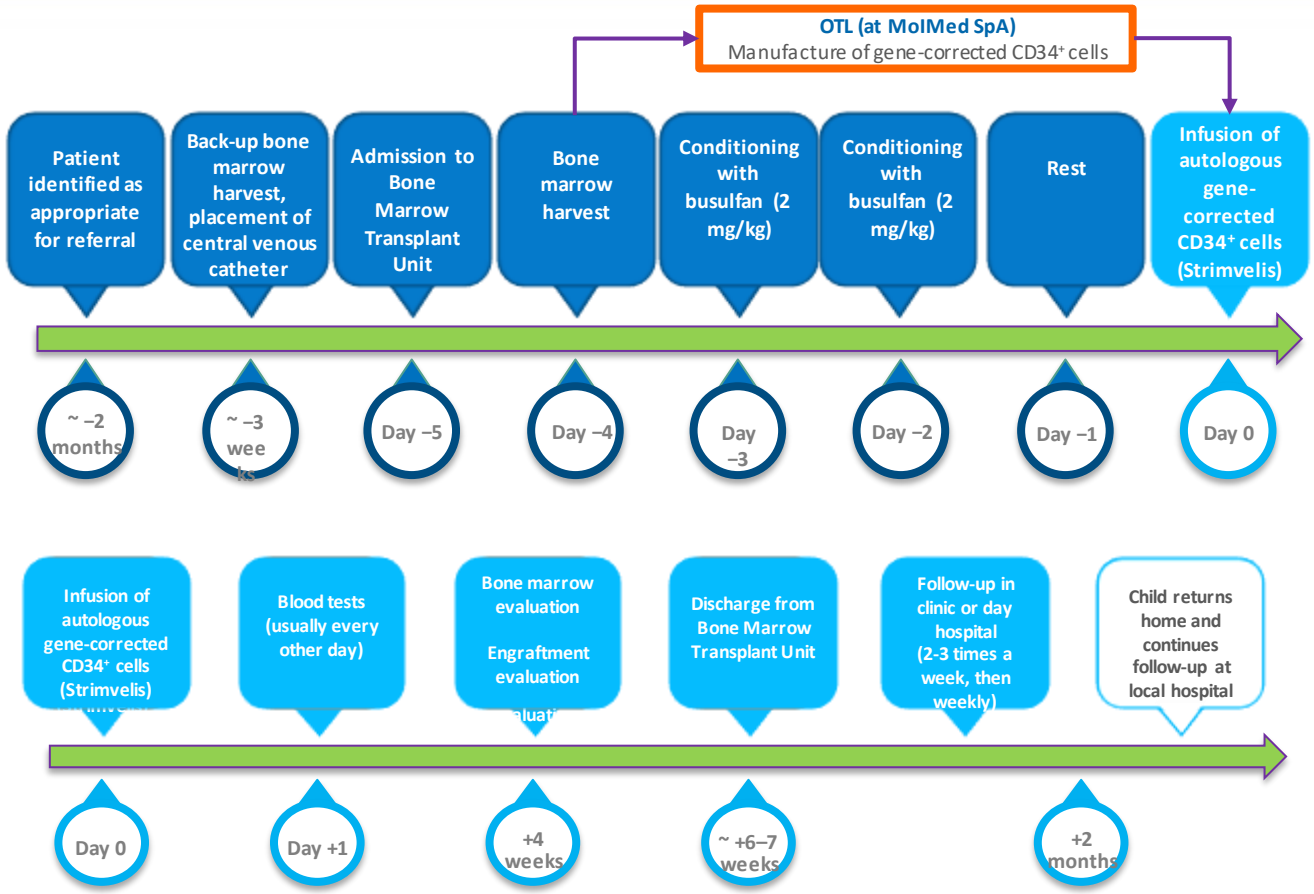
Strimvelis treatment path[1]





1st ex-vivo gene therapy registered in EU in **2016** for the **treatment** of patients with **severe combined immunodeficiency due to adenosine deaminase deficiency** (ADA-SCID), for whom no suitable human leukocyte antigen (HLA)-matched related stem cell donor is available

FRESH drug: stable for only 6 hours

*1. Aiuti A, et al. N Engl J Med 2009; **360**: 447-58; 2. Strimvelis Summary of Product Characteristics. June 2016.*

Paradigm shift to enable patient access to the therapy

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STRIMVELIS case study:

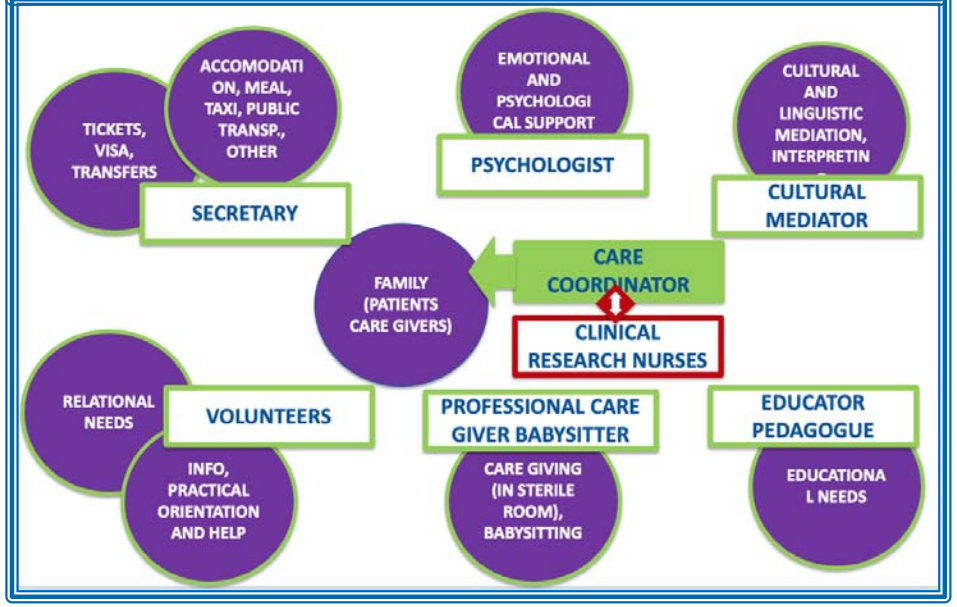
the patient has to travel, not the drug

How to fund treatment costs (i.e., drug/ clinical care)?

How to support patients/families in moving?

In-depth analysis on current EU Provisions for **PLANNED Treatments Abroad**

JUST LIKE HOME program



Summary of the Current EU Provisions for PLANNED Treatments Abroad - Differences

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	Social Security Regulations 883/04 and 987/09	Directive 2011/24/EU on patients' rights in cross-border healthcare:
General principles	• **Direct assistance** - The health services are **paid directly as if the patient is insured by social security system of that country.**	• **Indirect assistance** - **The patient have to pay** for treatments and then to **request a refund** in home country with proofs of payment. Refund will be based as if the treatment was provided in home country
Country variability	• **Regulati… requirem…** …ountries	• **Directive** = …nal **implement… national rules**
Need for prior authorisation	• **Yes,** prior… required (**S2 Form… …ent in the home… …hin a time limi…**	• **No**, for a wi… **authorisati… the rule**
Costs covered & Tariffs	• **Form S2 … …ugs) + clinical c… …linical services) … country** • **Logistics… …**ered and are managed case by case by the insurance, social assistance, etc.	• **Costs cove… … tariffs** (=> country to country variations). • **Logistics:** Travel and lodging typically not covered. • Not covered: Long term care, organ transplantation, public vaccination programmes
Eligible HC providers	• Covers **only public or private contracted with the National Health System healthcare providers** in the EU.	• Covers **all healthcare providers** in the EU, public or private

No money anticipated by the family > provided pre-authorised FORM S2 obtained

Patient has to pay in advance -> then reimbursed when back home

Key learnings from Strimvelis case study

- **Complex disease** requires **highly specialised clinical competences** not available in all the EU Countries

- **Complex therapies** requires concentration of knowledge, **specific logistic requirements,** training and **qualification of centers** not available in all the EU Countries

- **Move the patients across EU** to **the few highly specialized centers** could be ***safer*** for the patient and ***more cost-effective*** for the European system

- There is a ***need for a CENTRALISED EU APPROACH to ensure TIMELY patient access***

Time for ACTIONs!

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☆Leverage Strimvelis experience to facilitate ***TIMELY*** **Access** of Orphan ATMP for patients affected by rare/«ultra-rare» diseases

☆ Model at EU level cross-border access **PILOTs** to ensure delivery of high quality care for **ultra-rare/low prevalence diseases**

☆Consider all aspects of the drug/patient journey; **classification** of diseases, coordination of **assessment,** unified **funding,** common **selection criteria for centers,** harmonization of **evidence collection,** coordinated **patient management and monitoring**

☆Establish **cross-stakeholder EU partnerships** to support design and implementation of pilots at the local level

1990-2020

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COMMITMENT
CHALLENGES
FUTURE
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