Challenging path to bring a gene therapy to patients with an ultra-rare disease

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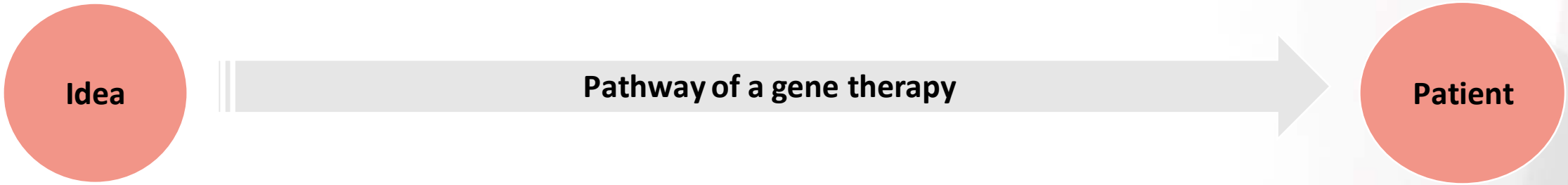
Introduction

- Immense scientific and clinical progress
- 2020: A year of significant growth in the regenerative medicine sector
- Clinical pipeline is robust: 1000+ companies and 1100+ clinical studies (>100 in phase 3)
- Thousands of patients are now benefitting from commercial regenerative medicines, and the impact of the early cell and gene therapies is dramatic
- Several late-stage products poised for approval, e.g. PTC's Upstaza for AADC
- Field moving also outside of oncology and to larger patient populations
- Sector expands manufacturing capabilities

Examples of Challenges along the way



R&D

- Hospital exemption legislation vary across member states, impacts incentives for innovation

Regulatory Approval Pathway

- Gap between regulators and HTA bodies in evidence criteria & use of RWE
- Status of Conditional Marketing Authorisations

Pricing and reimbursement

- Barriers to innovative payment models (e.g. annuity payments) across member states

Access to Patients

- Variability in the use of cross-border healthcare legal provisions across Member States

What does this mean for Umbrella patient groups?

- Not every (ultra) rare disease has a patient advocacy group – umbrella groups play a key role
- Gene therapies often treat rare and ultra rare diseases
- Ultra rare disease patients have an additional challenge:
  - Limited awareness of the disease due small patient number (as low as one or two for some countries)
  - Limited medical expertise and clinician involvement (specialised centre only)
  - Individual family or individual patient advocates without support from a patient organisation (limited knowledge, resources etc. to advocate for treatment)
- Umbrella groups can help addressing the following questions:
  - Is gene therapy understood by relevant stakeholders? – patients/caregivers, PAGs, clinicians, health authorities, HTA bodies, government decision makers
  - Is the (access) P&R process understood by the patient advocate/group (e.g. patient input into HTA)?
  - What is the process for accessing treatment, including access to treatment abroad?
  - What is the relations with the Specialised treatment centre and ERNs
- Difficult challenges and questions where umbrella groups can help!

Conclusions

- ATMPs such as gene therapies are a great innovation – adding to the arsenal of treatments for patients with unmet medical needs!
- Considering that 5 of the 13 ATMPs receiving marketing authorisation have been withdrawn for commercial reasons, it is essential to educate on the complexity of the ATMP innovation model
- It is important to foster an environment that is supportive of sustainable innovation. Otherwise, the consequences are restricted patient access to potentially curative therapies and a reduced incentive for manufacturers to further invest in this potentially life-changing medical technology
- We require a dialogue about the ATMP innovation model; it is time-consuming, costly and high risk. Much of the costs and risks are borne by the private sector, even though the public sector's contribution is important
- Umbrella Rare Disease Patient groups can assist patients with issues related to access to gene therapies and can advocate for ultra rare disease patients which perhaps have not full organisation to support them