# No time to wait: Can early access schemes work for gene therapies?

ISPOR EUROPE 2021 PANEL SESSION

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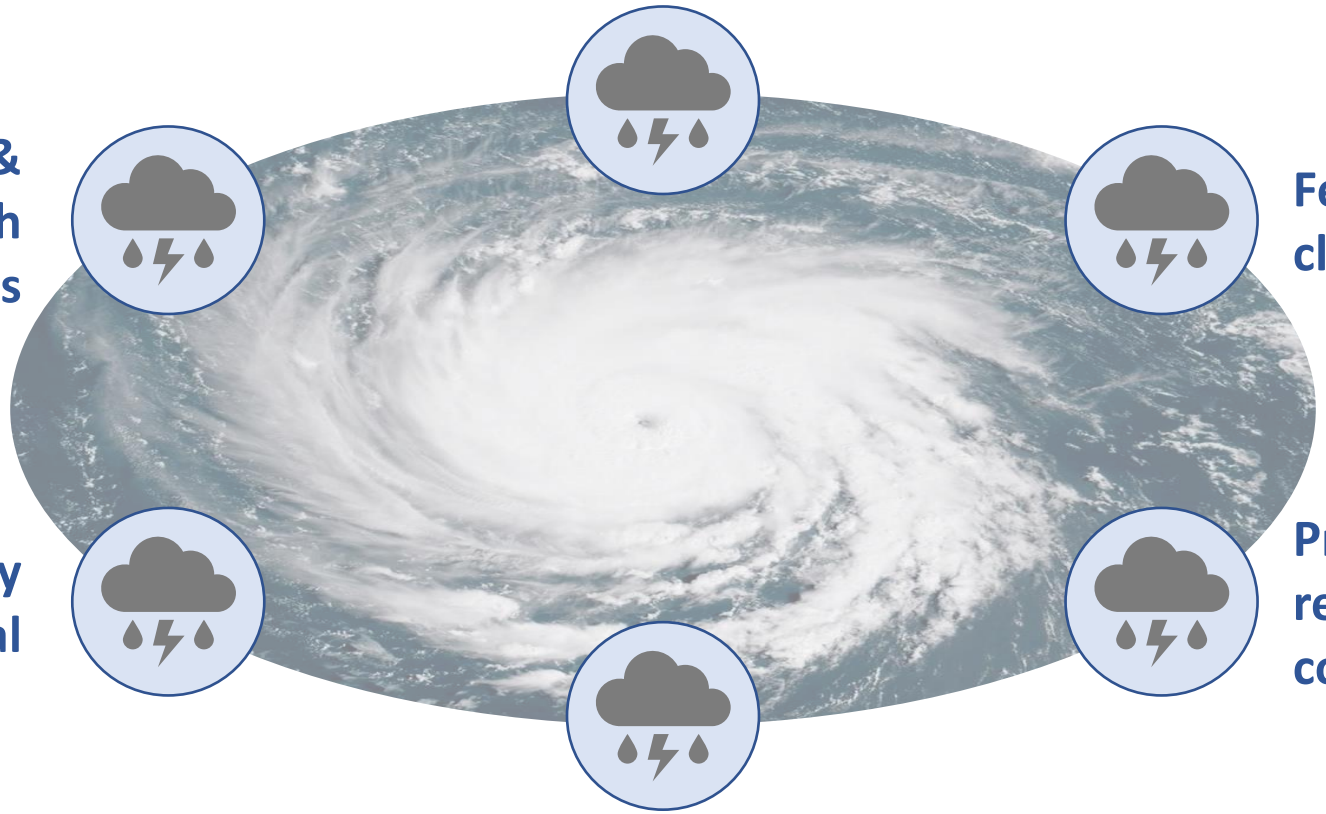
# A variety of precipitating events specific to innovative gene therapies can result in an early access "perfect storm"




ATMP: Advanced Therapy Medicinal Products.

# Sustainable early access solutions are needed for gene therapies because they are fundamentally different than their conventional therapy counterparts

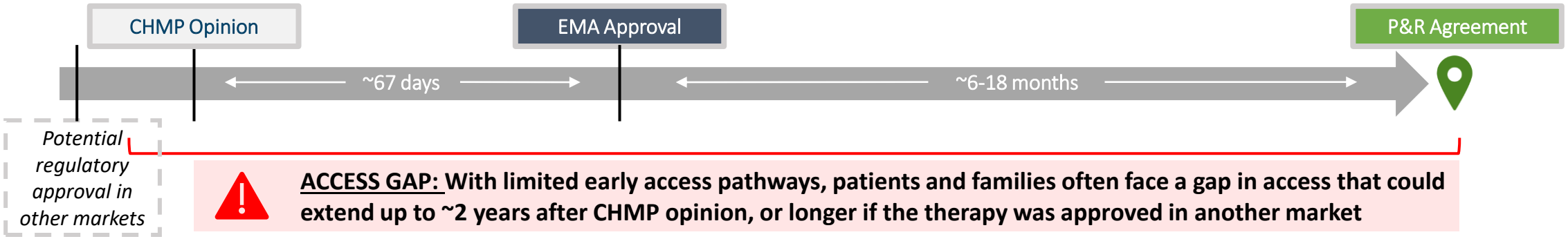
| | Conventional Therapies | Gene Therapies |
|---|---|---|
| RoA | Repeated, often chronic administration for months or years[1] | Usually **single-dose** or short-course administration[1] |
| MoA | Treatment targets symptoms of the disease but may not tackle underlying cause[1] | Designed to address the **root cause** with the potential to **halt disease progression** or even be curative[1] |
| Duration | Treatment must be continued for a sustained effect[1] | **One-time intervention** with **multi-year benefit**; potential for lifelong benefit[1] |
| Access | Value assessment processes established with traditional randomized controlled trials expected as gold standard[1] | Greater uncertainty in longer term outcomes and novel study designs **challenge value assessment**[1] |
| Payment | Payment models are designed with chronic administration of therapy in mind[2] | Single-dose treatment and higher upfront cost creates **challenges for usual payment models**[1,2] |

**Gene therapies are game-changing, but the access pathways are designed for conventional therapies; early access options are extremely limited, with very few pathways implementing funded early access pathways (e.g., France)**

RoA: Route of Administration;; MOA, mechanism of action. 1. Alliance for Regenerative Medicine. Getting Ready: Recommendations for Timely Access to Advanced Therapy Medicinal Products (ATMPs) in Europe. Available at: https://alliancerm.org/sector-report/market-access-report/. Accessed: June 2021; 2. European Federation of Pharmaceutical Industries and Associations (EFPIA). Finding solutions for access to new treatments in rare disease. Available at: https://www.efpia.eu/news-events/the-efpia-view/blog-articles/26092019-finding-solutions-for-access-to-new-treatments-in-rare-disease/. Accessed: June 2021.

# Because early access pathways are limited, families and patients seek access prior to formal P&R through crowdfunding, an inequitable and imperfect solution

## Timeline to Formal P&R




## CROWDFUNDING RISKS[1]

- Many families have resorted to public appeals for financial support (i.e., crowdfunding), even before formal regulatory approval
- Crowdfunding projects have been accelerated by the approval of highly expensive drugs (cell & gene therapies)
- While crowdfunding itself is not unethical, its use can have consequences that may influence perceptions of healthcare and how it is delivered

### Patient's privacy

**Disclosure / dissemination of personal information** (e.g., genetic information) about family members without their informed consent

### Uneven distribution

Undue influence on the **allocation of resources and inequitable access to finance** based on patients' social status and/or their existing social network

### Biased information

Patients and financial donors need **clear and unbiased information about the risks and realistic benefits** of the "crowdfunded" therapies

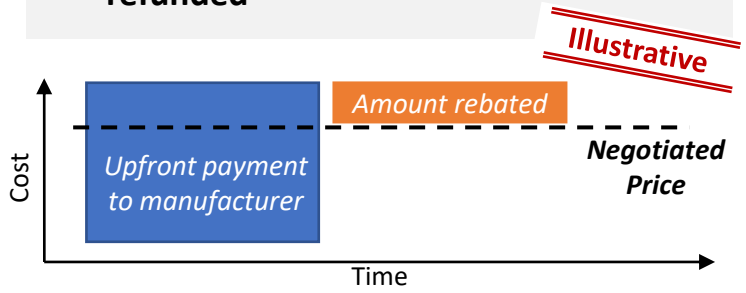
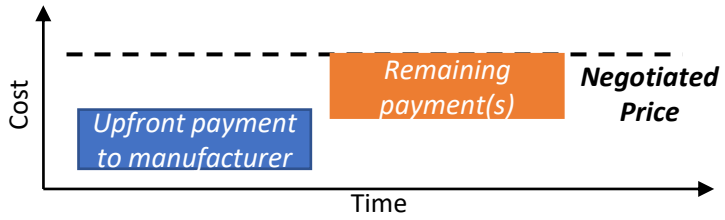
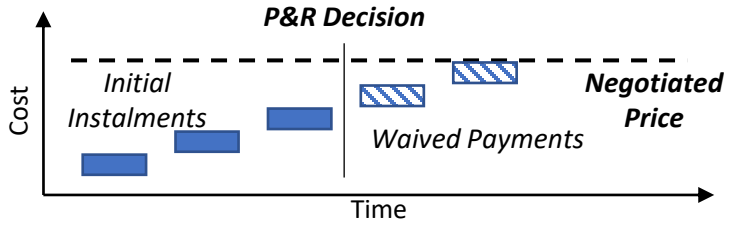
### Additional beneficiaries

Additional / external players can **take advantage of patients' needs** for an urgent treatment and **get benefit throughout the crowdfunding process**

1: Livingstone, et al. The Lancet Neurology. 2021 Oct; 20(10): 788-789. P&R: Pricing & Reimbursement; CHMP: Committee for Medicinal Products for Human Use; EMA: European Medicines Agency

# The ‘Access Gap’ can be addressed by changing local regulations or providing reassurance that early access will ultimately be based on the value-based price

***Potential Schemes to Address Access Gap***

| | 1 | 2 | 3 |
|---|---|---|---|
| | **Retroactive Rebates** | **Deferred Payments** | **Instalment Payments Waived after P&R Decision** |
| DESCRIPTION | • **Full payment** at time of administration<br>• After formal P&R agreement, **difference from the negotiated net price is refunded** | • **No or partial payment (instalment)** at time of administration<br>• **Remaining instalments deferred** until P&R agreement is reached | • Payment in **instalments over time**<br>• Any **remaining instalments** at the time of P&R decision are **waived** (i.e., reduced cost for early access patients) |
| |  |  |  |
| BENEFIT | **Institutional payers do not overpay** prior to P&R decision | **Reduced upfront cost** to address budget impact and no overpayment | **Accelerated P&R agreement** through cost savings |

**Early access schemes must ensure the continued integrity of the local pricing and reimbursement frameworks; some markets present legal barriers to early access, which will need to be overcome**

P&R: Pricing & Reimbursement.

# Requests for early access and crowdfunding are here to stay, unless healthcare system and all stakeholders work together to find better solutions

***<u>Policy Ask</u>: To establish paid, early access pathways for single administration gene therapies to enable timely treatment of patients with very high unmet need who cannot afford to wait.***

- **Single administration** gene therapies, with transformational outcomes will **increase the demand for immediate access solutions**
- **Early access funding pathways** (e.g., such as the system in France) will increasingly be needed to provide timely solutions for patients and families in the future
- **Engaging and collaborating with policymakers** will be critical to design effective and timely solutions