

“You can’t have your cake.....
..... and eat it”
English Idiom

“You can’t have your **therapy**.....
..... and **use it**”

What is the problem?

Therapy Development

- OMP Legislation
- New innovative therapies Gene and cell therapies
- National / International availability of specialised centres
- EMA PRIME /Conditional Approval
 - New Pilots
- National Early Access programmes
- Multiple possibilities for payment models

Access Limitations

- High Production Costs
- Ineffectual Cross Border / S2 pathway
 - Assessed in patients country
- Systems inabilities to carry out monitoring for payment models
- Inappropriate use of cost effective /Budget impact models
- No mechanism for multi annual payment for 1 off therapy
- Simple discounts
- Crowdfunding

Result: 6 Gene or cell therapies left the European market in the last 2 years for Non Medical Reasons

Reasons for Optimism

- Academic Non-for profit academic intuitions
- French Payers requesting national legislation change to allow multi-year agreements
- Dutch Ministries employing Managed Accessed Agreement personnel
- EU Joint Procurement of Vaccines
- Beneluxa approach to two gene therapies promising with earlier access than before
- Ireland and Italy having effective screening programs to identify patients early

- Need to find ways to expand and develop successes across Europe

Europe-wide group launched to boost access to life-saving gene therapies

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AGORA 
Access to Gene Therapies for Rare Diseases