Challenges in European Commercial ATMP development

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Escher-ATMP Project

Overall Aim:

Examine factors associated with successful ATMP development and commercialisation in Europe.

Work package 1

Survey: Provide overview of product and developer characteristics and identify experienced challenges.

Work package 2

Interviews: Substantiate the observed findings from WP1 by identifying challenge root-causes.

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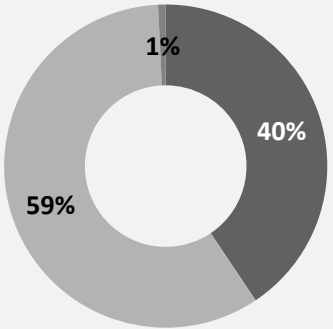
ATMP Landscape in Europe

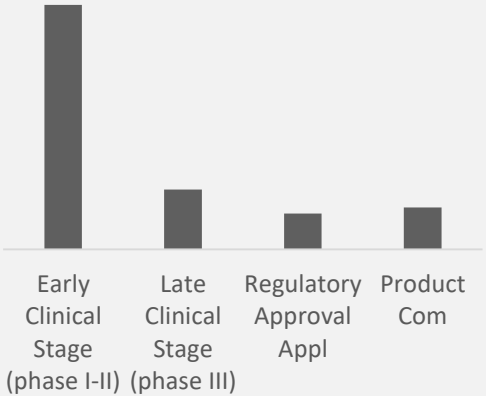
271 EU developers

In 2016
65% of developers are SMEs,
35% are large developers.

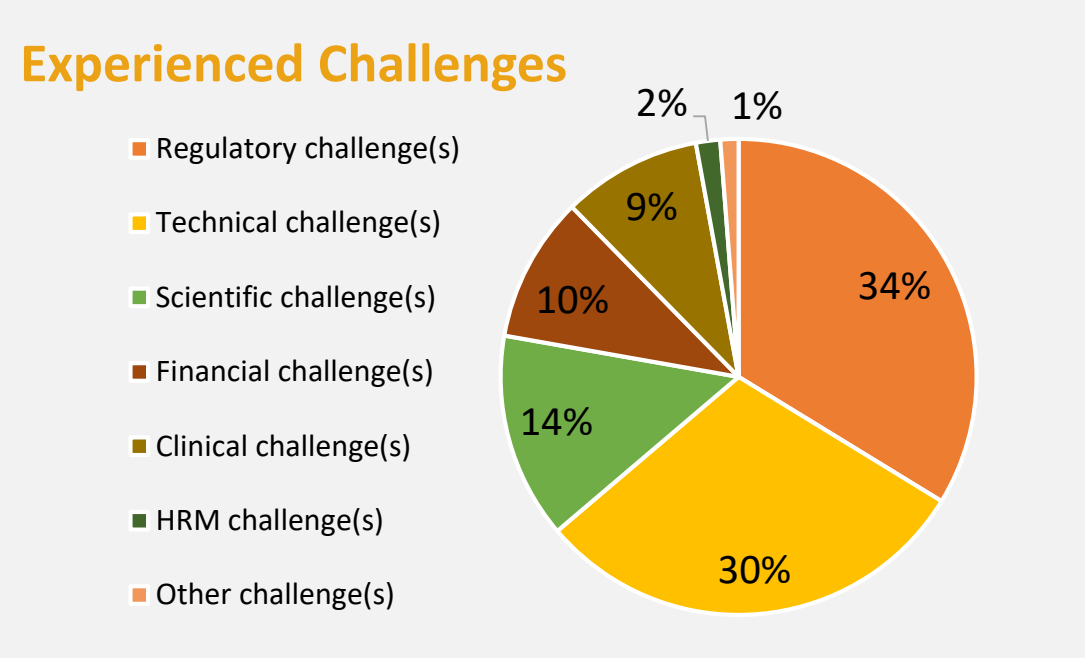
ATMP Type



Developer Development Stage



Survey Results



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Survey Results

Top 3 Experienced Challenges

1. 16% **Country Specific Requirements**
2. 15% **Product Manufacturing**
3. 8% **Clinical Trial Design**

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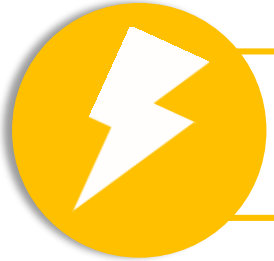
Combining Survey Results with Interviews

Many different challenging identified in commercial EU ATMP development.
Two elements causing many hurdles are:



The disconnect between European (high level) regulation and national hurdles.

Capabilities and business model at the start have effect throughout ATMP development process.

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Disconnect between European (high level) regulation and national hurdles

European efforts helped ATMP development.

Local interpretation and execution lead to many additional information requests.

Difference in national interpretations:

- GMO legislation
- Clinical Trial Applications
- Good Manufacturing Practice
- Hospital Exemption

Small companies do not have the resources to address country specific requirements.

Data request burden drives launch preference of global developers.

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Capabilities and business model at the start have effect throughout ATMP development proccess.

Include expertise and business goals from the start

Realistic and detailed business model

Payers/HTA need to join developer interactions

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Concluding: Escher-ATMP project

- EU ATMP landscape is in early stages.
- European efforts well received by developers. National authorities have not fully matured.
- CTA, GMO and GMP harmonization between member states is needed, also to keep competing with other international markets.
- ATMP development is more complex than more traditional drug development. Include regulatory and manufacturing expertise and have a detailed business model, increase development success.

Reference: Ten Ham et al, Challenges in Advanced Therapy Medicinal Products Development: A survey amongst companies in Europe; Molecular Therapy – Methods & Clinical Development; 2018

By Renske ten Ham, MSc - Division of Pharmacoepidemiology and Clinical Pharmacology - Utrecht University

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Concluding: Take home message

Stakeholder specific recommendations

European Regulators: Are shaping European ATMP landscape, include local authorities and payers/HTA in the process.

Local Regulators: Need to bridge knowledge gap between national and European authorities.

SMEs: Early on include GMP grade standards, regulatory and quality knowledge and build realistic business plan. Partnerships with experienced party to accelerate development and funding.

Large companies: Same steep ATMP learning curve as SMEs but more experience in pharmaceutical development. Partner with SMEs to fill pipeline and accelerate innovation.

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