HTA201

Access Challenges of Advanced Therapy Medicinal Products in Europe

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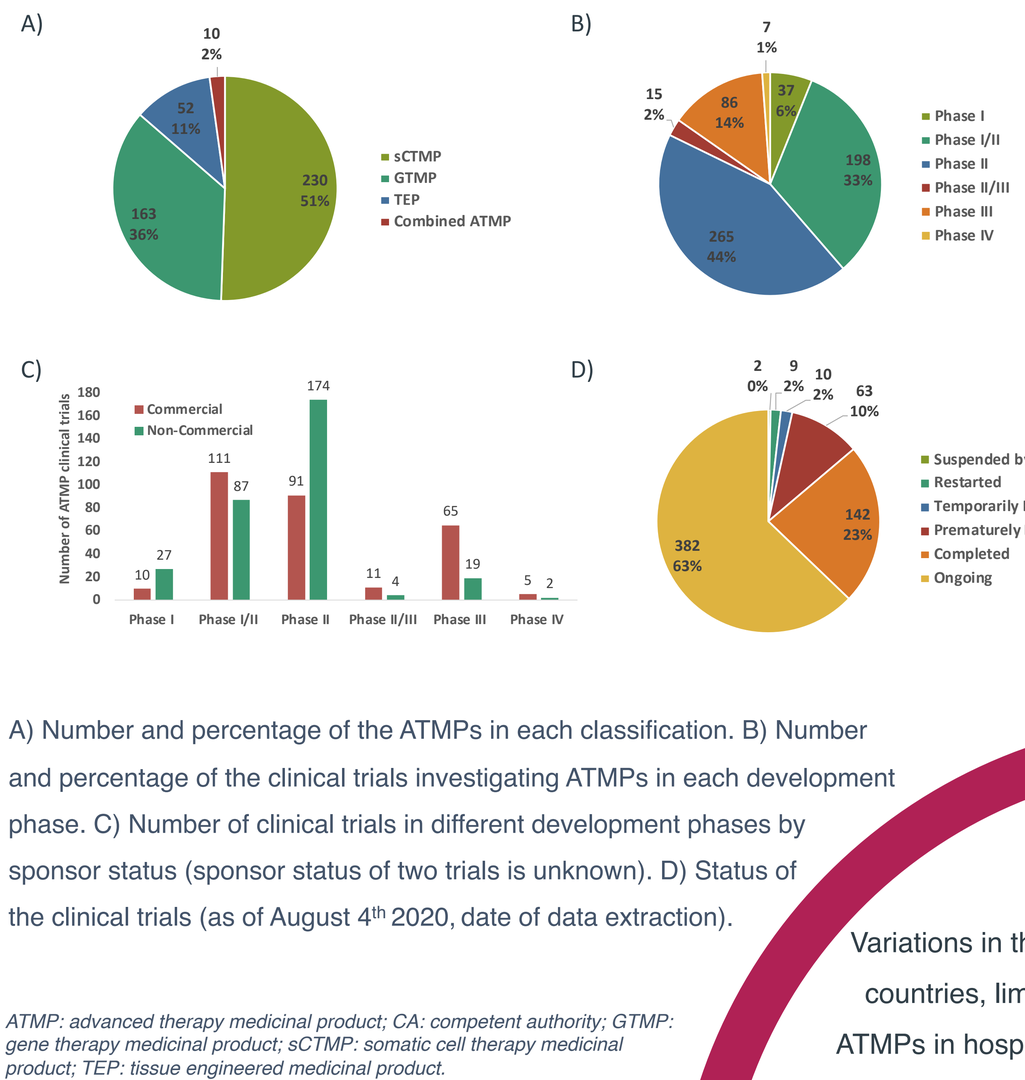
INTRODUCTION: Advanced therapy medicinal products (ATMPs) are innovative therapies with potential to cure or significantly transform the health status and quality of life of patients with conditions that until recently were considered uncurable. Due to the potential high value of these therapies, questions around the funding and affordability are emerging. In order to determine the real dimension of this concern, it is important to quantify the number of patients eligible for ATMP treatment in the near future.

OBJECTIVES: To determine the number of ATMPs either recently approved or likely to be approved in Europe until 2024 and to estimate the number of patients that could be eligible for treatment with each product in the EU27+UK.

1 607 clinical trials investigating 455 ATMPs were registered in EudraCT database from 2010

Characteristics of the ATMPs and their corresponding clinical trials registered since 2010 in EudraCT database



A) Number and percentage of the ATMPs in each classification. B) Number and percentage of the clinical trials investigating ATMPs in each development phase. C) Number of clinical trials in different development phases by sponsor status (sponsor status of two trials is unknown). D) Status of the clinical trials (as of August 4th 2020, date of data extraction).

ATMP: advanced therapy medicinal product; CA: competent authority; GTMP: gene therapy medicinal product; sCTMP: somatic cell therapy medicinal product; TEP: tissue engineered medicinal product.

2 31 ATMPs have the highest probability to reach the market within the next 3 years

608 CTs
455 ATMPs
registered in EudraCT since 2010

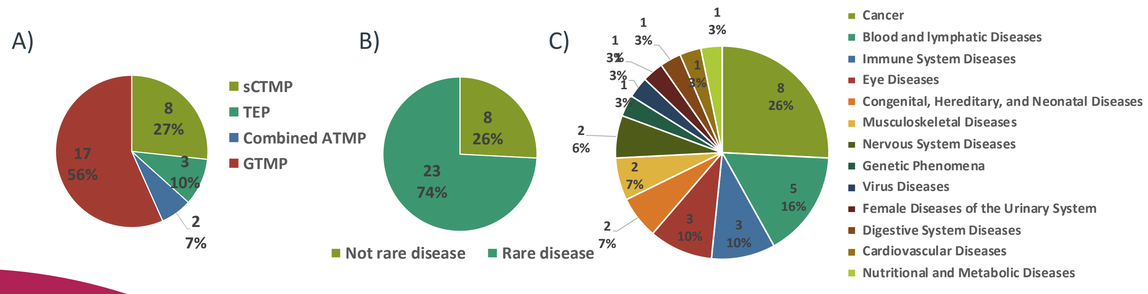
Exclusion of:
- Clinical trials in phase I and phase I/II
- Clinical trials by non-commercial sponsors
- Clinical trials that prematurely ended or were suspended by CA

126 CTs
86 ATMPs
in advanced stage of development

Exclusion of:
- Failed clinical trials or ATMPs with no publicly available information on their investigation status for the last 2 years
- Clinical trials that are not registrational or that are not expected to lead to launch of the product before the end of 2024

31 CTs
31 ATMPs
with high potential to enter European markets before 2024

Characteristics of the ATMPs and their corresponding clinical trials with high likelihood to reach the market before the end of 2024



A) Number and percentage of the ATMPs in each classification. B) Number and percentage of clinical trials investigating rare versus not rare diseases (defined by the sponsor). C) Classification of ATMP clinical trials by disease areas under investigation.

ATMP: advanced therapy medicinal product; GTMP: gene therapy medicinal product; sCTMP: somatic cell therapy medicinal product; TEP: tissue engineered medicinal product; Data analyzed in June 2022

CONCLUSIONS:

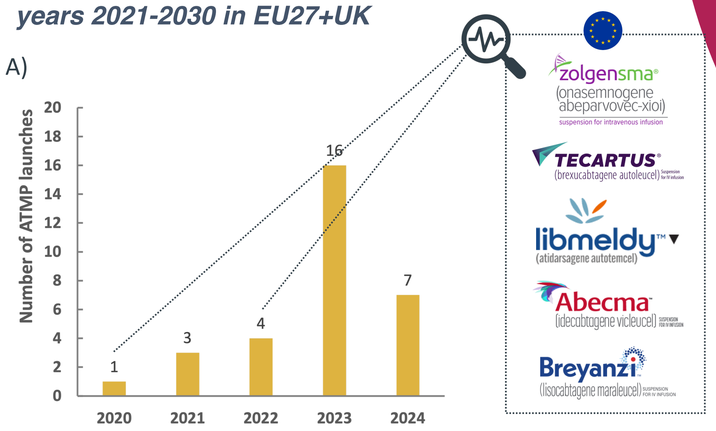
Variations in the ATMP therapies reimbursement between countries, limited eligibility for extra funds for managing ATMPs in hospitals, and commercially driven withdrawals of some ATMPs fuel uncertainties in ATMP access in Europe.

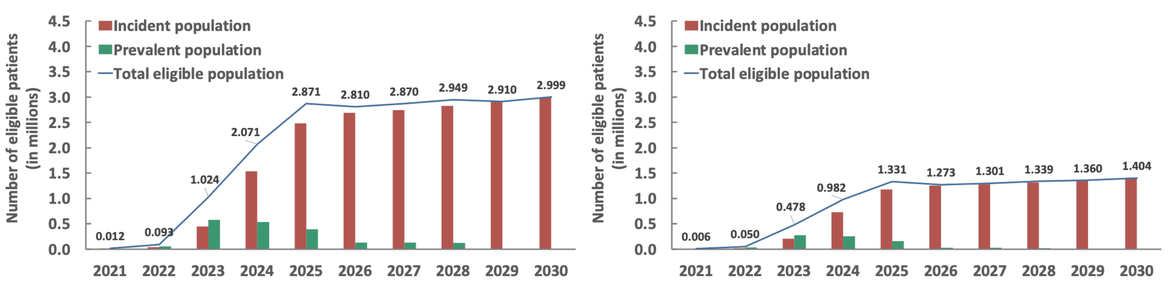
Our study shows that the pipeline of ATMPs is expanding beyond rare indications and about 20 million people could be eligible for ATMP therapy in EU27+UK until 2030.

This confirms the concerns raised in the recent years about the affordability of these therapies for European healthcare systems, but more importantly, highlights **the urgent need for the implementation of adequate infrastructure and payment schemes adapted to the unique value offered by this new type of medicines.**

3 About 20M people in EU could be eligible for ATMP therapy

Number of ATMP launches and number of patients eligible for treatment with upcoming ATMPs in the years 2021-2030 in EU27+UK





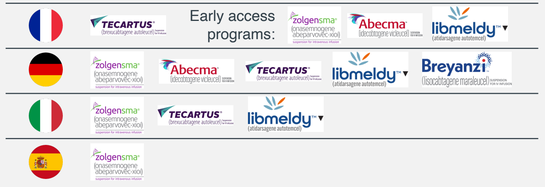
A) Number of ATMPs coming to the market each year assuming 100% probability of launch success. B) & C) Incident, prevalent, and total population potentially treated with ATMPs by year, B) assuming that 100% of eligible patients will be treated, C) assuming a limited and specific peak market share for each ATMP (i.e., not all eligible patients will be treated with the product) by qualitative analysis of the extrinsic (e.g., competitive landscape) and intrinsic factors (e.g., disease modifying potential) influencing future product access.

ATMP: advanced therapy medicinal product; Data analyzed in June 2022

4 ATMPs face access challenges in Europe

- 20 ATMPs have been approved in Europe until June 2022.
- The **uncertainty on the value, high cost, and one-shot nature** of many ATMPs challenge the classical pricing and reimbursement process.
- Limited adaptation of the HTA process has been observed for the assessment of ATMPs.
- Reimbursement of ATMPs varies among countries reflecting the different assessment methods and the financial tools used to manage the risk.
- Already **7 ATMP therapies have withdrawn for commercial reasons** from European markets.

Reimbursement of recently approved ATMPs



ATMP: advanced therapy medicinal product, HTA, health technology assessment; Data analyzed in June 2022

METHODS:

- We reviewed ATMPs clinical trials registered in EudraCT database and systematically selected products approved or likely to be approved in Europe between 2020 and 2024.
- We estimated the size of the target population for each product.
- We used publicly available data sources to estimate the probability of the launch of each product and the epidemiology of each condition.

ABOUT THE AUTHORS:

LatticePoint has been working with clients for decades in a range of therapeutic areas. Our clients trust our world-class team of market access leader's experience in the sciences, industry, venture capital and negotiations assess your product in the marketplace.

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