Translating Innovation into Access for ATMPs
3rd EU-Innovation Network Multi-Stakeholder Meeting

Industry Perspective

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FARMINDUSTRIA

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Farmindustria is the **Association** of the **Italian Pharmaceutical Companies**

It is a member of the Italian National Federation of Industry (Confindustria), the European Pharma Federation (EFPIA) and the World Pharma Federation (IFPMA)

Over **200 member** companies
40% of which are nationally-owned
60% foreign-owned

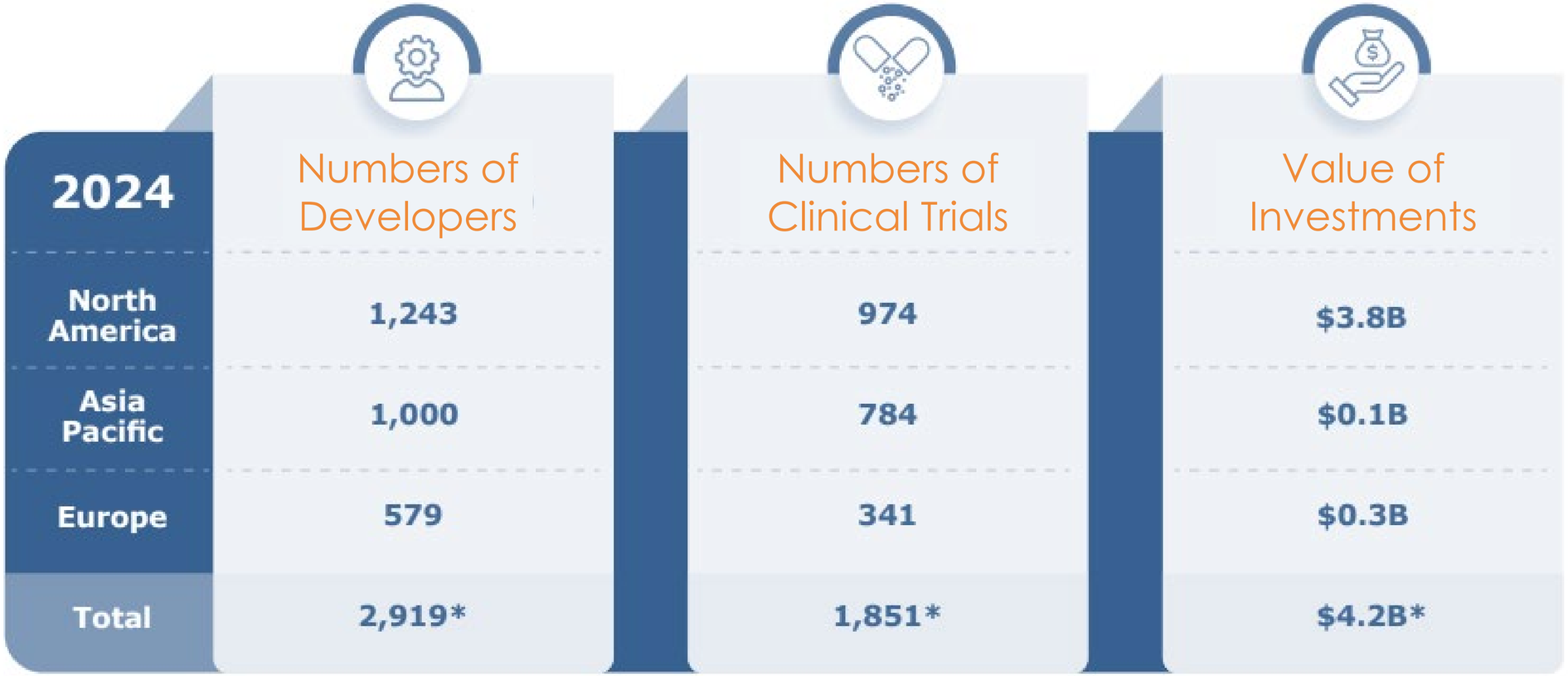
We represent about **95%** of the sector in terms of turnover, exports, investments, employment

CONFINDUSTRIA

efpia
European Federation of Pharmaceutical Industries and Associations

IFPMA
International Federation of Pharmaceutical Manufacturers & Associations

ATMPs Sector: Global Outlook

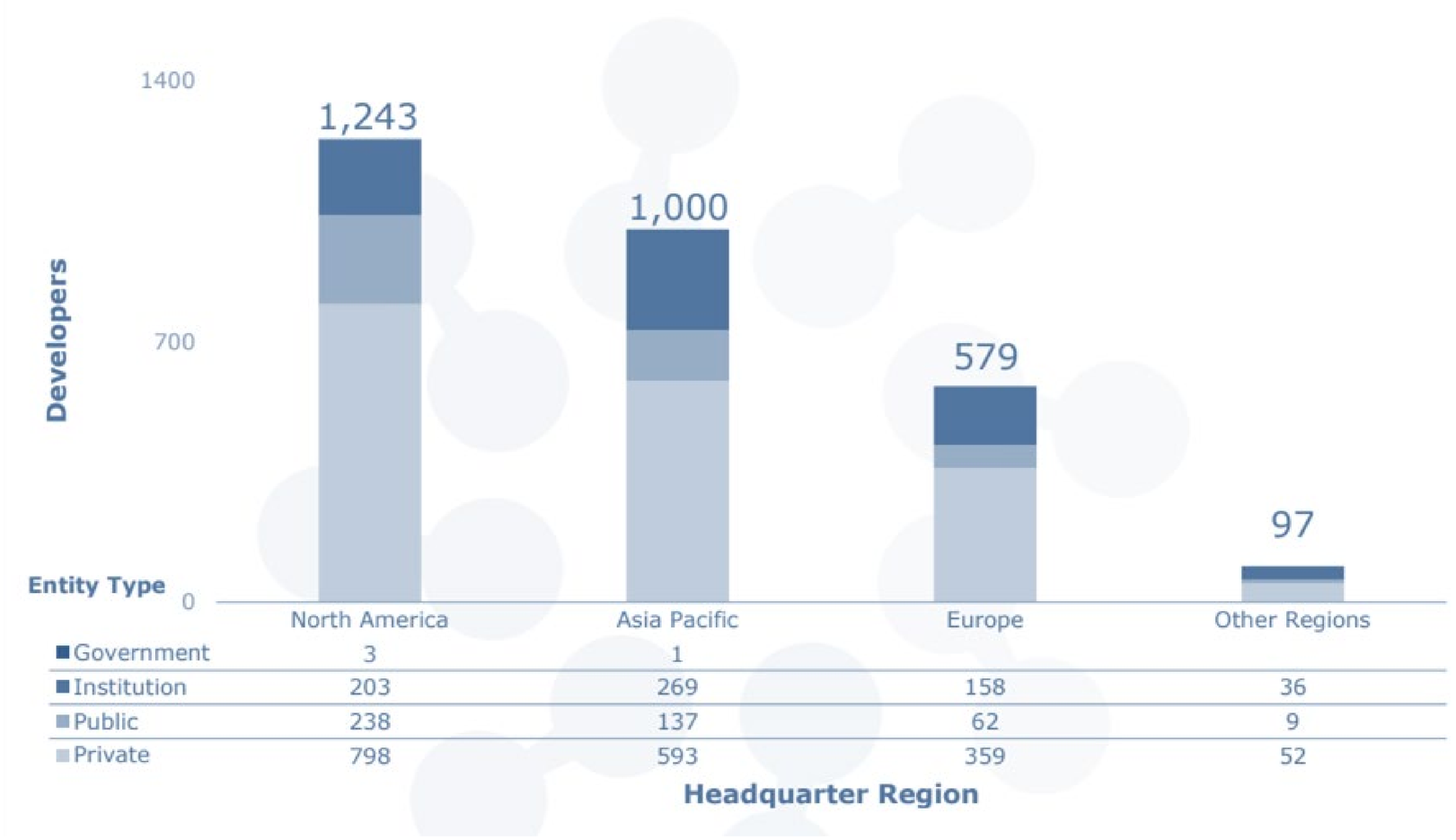


2024	Numbers of Developers	Numbers of Clinical Trials	Value of Investments
North America	1,243	974	$3.8B
Asia Pacific	1,000	784	$0.1B
Europe	579	341	$0.3B
Total	2,919*	1,851*	$4.2B*

*Totals refer to unique quantities and includes data from other regions not shown

Source: Alliance for Regenerative Medicine – Sector snapshot (August 2024)

Developers by Headquarter Region and Entity Type



INSIGHTS

North America Dominance
1,243 developers positioning North America as the leader, driven by private (798) and institutional (203) contributors.

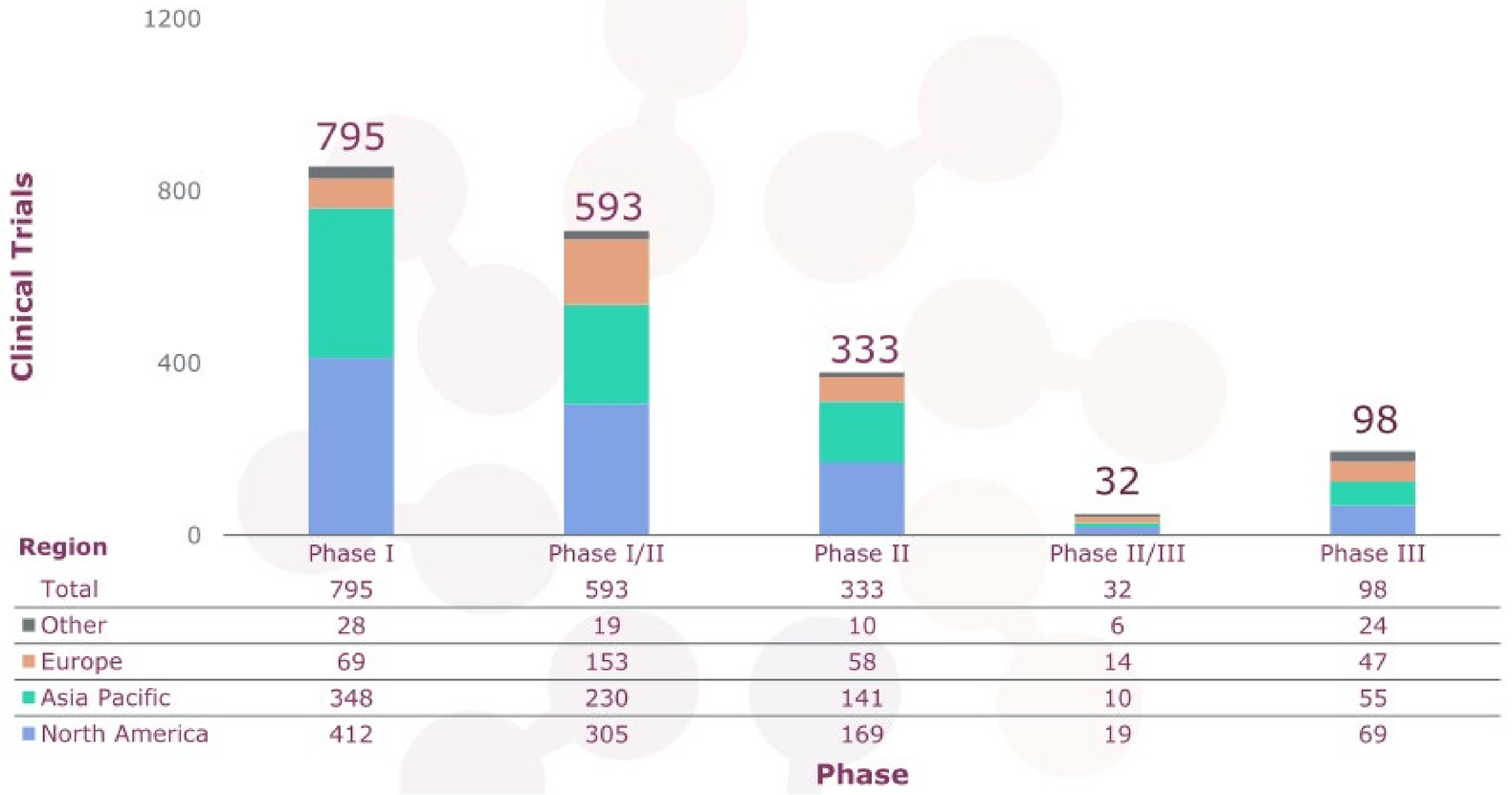
Rapid Growth in Asia Pacific
With 1,000 developers, the region shows strong momentum, led by private (593) and institutional (269) entities

Europe's Limited Presence:
Europe has 579 developers, primarily private (359), but trails behind North America and Asia Pacific, indicating room for growth

1. Within-graph labels represent headquarter region totals
2. Please see "Methodology Notes" for additional details

Source: Alliance for Regenerative Medicine – Sector snapshot (August 2024)

Ongoing Clinical Trials by Phase and Region



INSIGHTS

Phase I Dominance
North America leads in Phase I clinical trials with 412, making up more than half of the global total of 795

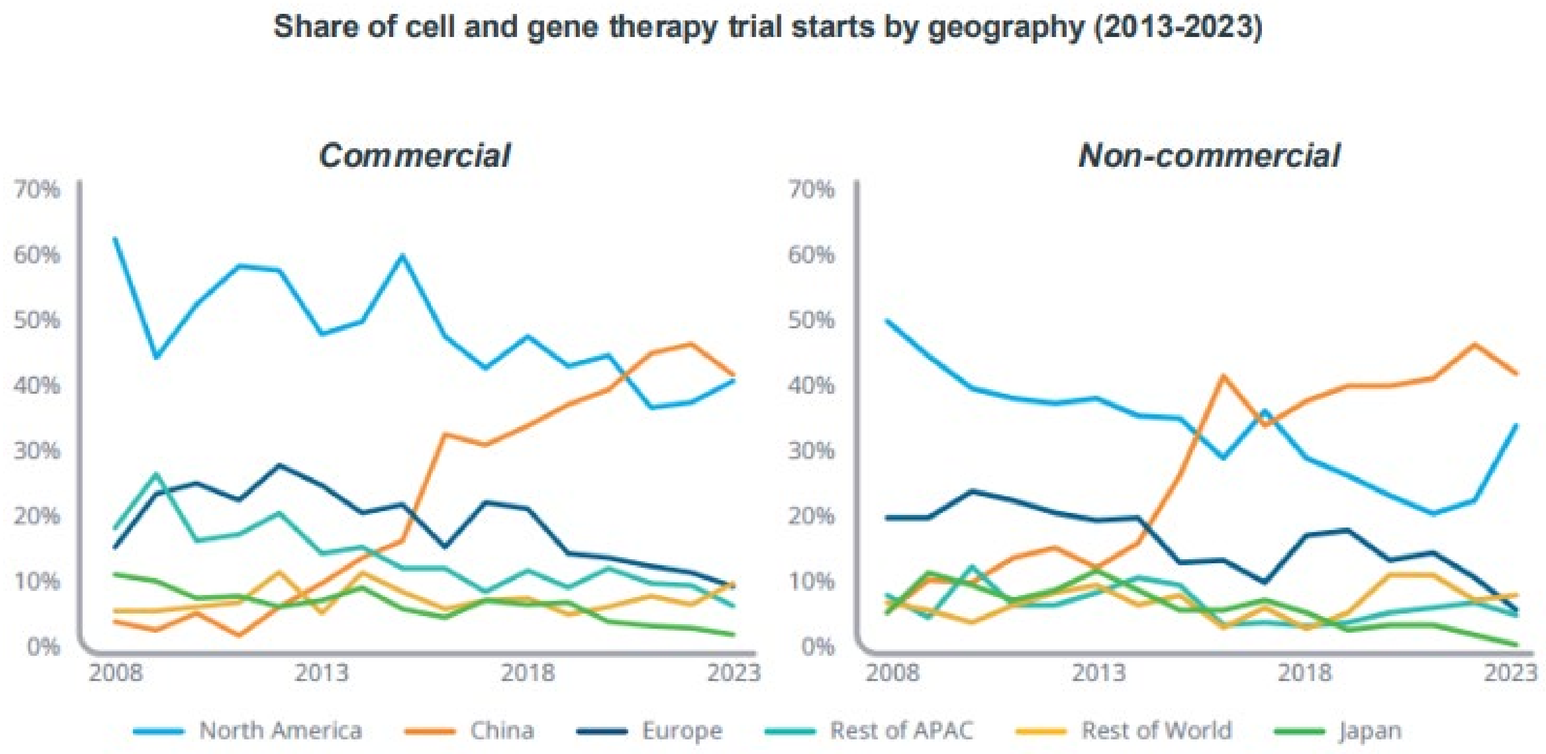
Strong Phase I/II in Asia Pacific Asia Pacific shows significant activity in Phase I/II trials, contributing 230 out of 593 trials globally

Europe's Steady Yet Limited Role
Europe demonstrates notable involvement in clinical research across all stages but remains behind other regions in terms of participation

1. Within-graph labels represent phase totals
2. Clinical trials take place in multiple regions; sum of regional totals will not be equivalent to phase totals

Source: Alliance for Regenerative Medicine – Sector snapshot (August 2024)

Europe's Share of Cell and Gene Therapy Trials Has Decreased Since 2013, Whilst China Has Experienced Rapid Growth in the Last Decade



INSIGHTS

The growth in China may be due to a favorable regulatory environment, funding availability, and strategic focus on C&GT

The US share of C&GT trials decreased from 2014 to 2022 but remains the second-largest region for both commercial and non-commercial trials

Since 2021, non-commercial C&GT trials have notably increased in the US, indicating a renewed focus on this area

Source: QVIA | EFPIA-VE | Assessing the Clinical Trial Ecosystem in Europe | Final Report | August 2024

Combined Factors Have Positioned China as the Global Leader in Clinical Trials for Cell and Gene Therapy

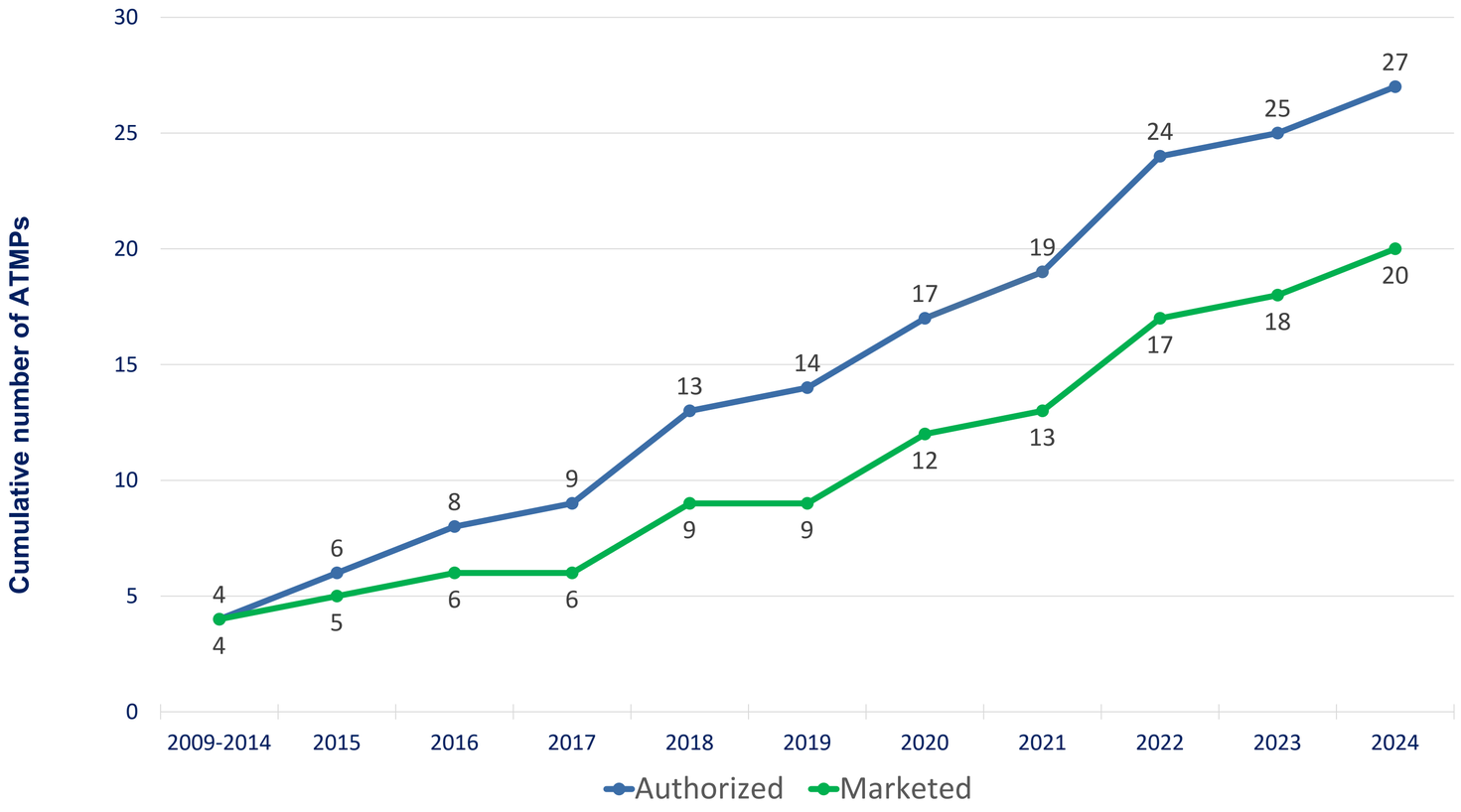
FAVORABLE REGULATORY ENVIRONMENT: the Chinese government has introduced policies that support R&D and Manufacturing in advanced therapies. For example, the "Made in China 2025" initiative aims to strengthen domestic manufacturing, including the production of innovative therapies like CAR-T

AVAILABILITY OF FUNDING: between 2018 and 2021, Chinese companies specializing in cell and gene therapy raised $billions. The influx of capital has accelerated research and development, accelerating the market entry of new therapies

STRATEGIC FOCUS ON C&GT: China has a strong strategic focus on advanced therapies, with programs like "Healthy China 2030" promoting innovation in the pharmaceutical and healthcare sectors. Additionally, China has invested in human capital, encouraging the return of Chinese scientists trained abroad to lead local research efforts

Source: Tracking the rise of CAR-Ts in China: the dawn of an immunotherapy superpower? Pharmaceutical Technology (May 27,2022)

Historic Yearly Marketing Authorizations for Atmps in UE



INSIGHTS

Today there are **20** ATMPs on the market in Europe, of which **14** are Orphan Drugs

These 20 ATMPs have a total of **30** therapeutic indications

18 are Gene Therapies (of which **6** are CAR-Ts), **3** are Somatic Cell Therapies, and **2** are tissue engineering products

Currently, no ATMP combined with a medical device has obtained marketing authorization

74% of ATMPs authorized are marketed

51% of ATMPs were authorized in the past 5yrs alone

Source: Source: EMA, CAT quarterly highlights and approved ATMPs (August 2024)

The Reimbursement Rate Is 30% Across All Countries Included in the Survey Which Is the Lowest Class

STATUS OF ATMP* REIMBURSEMENT
(2014-2021)

INSIGHTS

Excluding withdrawals, average reimbursement rate is **30%** across all countries included in the survey

No product has been reimbursed in all countries (maximum = **85%**); and there is no country that has reimbursed all ATMPs to date (maximum = DE, **95%**)

Source: EMA, CAT quarterly highlights and approved ATMPs; IQVIA WAIT indicator (2014-2022); ; * defined as per EMA definition of 'medicines for human use that are based on genes, tissues or cells'
*Their unavailability is due to market withdrawal (n=2) or lack of MA renewal (n=2)

Source: IQVIA

Is the Revision of the Pharmaceutical Legislation Going in the Right Direction?

CONSEQUENCES OF THE CAT'S ROLE ADJUSTMENT

Strategic Implications

The EU Commission's proposal for a reduction in the CAT's role may conflict with the need for specialized oversight in a rapidly evolving ATMP landscape, and it remains uncertain whether this change will lead to any reduction in authorization timelines.

Risk of Siloed Assessments

The Committee currently provides comprehensive, cross-disciplinary evaluations of ATMP projects, integrating CMC, non-clinical, and clinical assessments. Abolishing the CAT and dispersing its expertise among CHMP and working parties may lead to siloed reviews, compromising the integrated assessment essential for complex ATMPs

Cross-Fertilization of Expertise

The CAT facilitates knowledge sharing among all 27 EU Member States, enhancing national competencies and making more countries attractive for conducting clinical trials.

Is the Revision of the Pharmaceutical Legislation Going in the Right Direction?

EMA Fees for Scientific Advice

Starting from January 1, 2025, the 65% reduction on EMA fees will no longer be available. This revision removes the incentive previously available to all ATMP developers, reserving the benefit solely for SMEs and academic/non-profit entities, resulting in a disadvantage for the industry

Hospital Exemption

Clarify the interpretation of 'non-routine basis' to ensure a hospital exemption approval is only granted in cases when no authorised therapeutic alternative or clinical trial could satisfy the specific needs of the patient

Potential Challenges from SoHO Regulation and ATMP Interplay

Challenges from SoHO Regulation for Allogeneic Cell-Based Products: the upcoming SoHO Regulation may create problems for developers due to persistent national differences in qualifying starting biological materials (donor procurement), affecting both EU-collected and imported materials

Concerns Over Shift in Evaluation Competence: the responsibility for assessing borderline products is moving from the CAT to the SoHO Coordination Board, mainly composed of transplantologists and transfusion experts lacking this background.

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Thank you

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