Italian ATMPs: just an academic story?

Baila, Stefano[1]; Ceradini, Francesca[2]; Mazzaracca, Rachele[2];
[1]Network Officine Terapie Avanzate (NOTA-aps) - Italy;
[2]Osservatorio Terapie Avanzate (OTA) - Italy.

ABSTRACT

Italy has historically played a pioneering role in the field of Advanced Therapy Medicinal Products (ATMPs), laying the foundations for some of the most significant clinical milestones globally. Eleven and ten years ago, respectively, Europe approved the first stem cell-based therapy (tissue-engineering product) and the first ex vivo gene therapy. Both were developed in Italy, underscoring the excellence of Italian academic research and the country's translational potential in the ATMP field. The aim of this poster, born from the collaboration between Osservatorio Terapie Avanzate (OTA) and the Network Officine Terapie Avanzate (NOTA-aps), is to illustrate where Italy stands today in the ATMP landscape and what awaits us in the future. From 2009 to the present 33 ATMPs have been approved in Europe, 10 have been withdrawn from the market while 23 remain available; 17 of these therapies have been approved in Italy and 16 out of 17 are currently reimbursed by the National Health System. Access to these innovative but often highly expensive therapies has been made possible through various reimbursement models implemented by the Italian regulatory agency (AIFA). Italy also boasts a remarkably strong manufacturing capacity in the ATMP segment: four of the globally approved therapies have been produced in Italian facilities. However, the Italian ecosystem exhibits a unique dichotomy. While there is a relative shortage of private biotech companies and start-ups capable of driving late-stage commercialization, the country is characterized by an exceptionally strong presence of non-profit facilities. Currently, over a dozen academic and hospital-based GMP facilities are authorized by AIFA for the production of advanced therapies, acting as the primary engine for early-stage development and clinical trials. This also reflects the so-called "European paradox": strong scientific output that does not translate into an equivalent level of clinical trials and industrial development. To address this gap, the European Commission has presented the proposed Biotech Act. The Italian challenge will be to keep pace with European ambition, fostering greater involvement of the ecosystem at the commercial level and strengthening the synergy between the robust non-profit research sector and the pharmaceutical industry. This will be crucial not only for expanding the national manufacturing capacity but also for supporting the entire lifecycle of new drug development, ensuring that the therapies of tomorrow, much like those of the past, can continue to be developed and available in Italy.

CURRENTLY APPROVED ATMP IN EUROPE AND ITALY

(LAST UPDATED ON 13 OCTOBER 2026)

	NAME	MARKETING AUTHORIZATION HOLDER	THERAPEUTIC AREA	FIRST EU APPROVAL	MARKETING AUTHORIZATION IN ITALY	MADE IN ITALY
GENE THERAPY	**Imlygic®**	Amgen Europe	**inoperable melanoma**	December 2015	✗	
	Strimvelis®	Fondazione Telethon ETS	**ADA-SCID**	May 2016	July 2016	R&D, production, administration, vector production
	Luxturna™	Novartis Europharm Lim.	**hereditary retinal dystrophy**	November 2018	December 2020	
	Zolgensma®	Novartis Europharm Lim.	**SMA** (intravenous)	May 2020	March 2021	
	Libmeldy™	Orchard Therapeutics	**MLD**	December 2020	March 2022	R&D, production, administration, vector production
	Upstaza™	PTC Therapeutics	**AADC deficiency**	July 2022	November 2023	
	Hemgenix®	CSL Behring	**hemophilia B**	February 2023	July 2025	
	Vyjuvek®	Krystal Biotech Netherlands	**DEB**	April 2025	✗	
	Waskyra™	Fondazione Telethon ETS	**WAS**	January 2026	✗	R&D, production, administration, vector production
	Adstiladrin®	Ferring Pharmaceuticals A/S	**NMIBC** (intravesical suspension)	May 2026	✗	
	Itvisma®	Novartis Europharm Lim.	**SMA** (intrathecal)	July 2026	✗	
CAR-T GENE THERAPY	**Kymriah®**	Novartis Europharm Lim.	**ALL, DLBCL and FL**	August 2018	August 2019	
	Yescarta®	Kite Pharma EU	**HGBL, DLBCL, PMBCL and FL**	August 2018	November 2019	
	Tecartus™	Kite Pharma EU	**mantle cell lymphoma and ALL**	December 2020	March 2022	
	Abecma®	Bristol-Myers Squibb / Celgene	**multiple myeloma**	August 2021	January 2024	
	Breyanzi®	Bristol-Myers Squibb / Celgene	**DLBCL, HGBCL, PMBCL, FL, MCL**	April 2022	January 2024	
	Carvykti™	Janssen-Cilag International	**multiple myeloma**	May 2022	February 2026	
	Aucatzyl®	Autolus GmbH	**ALL**	July 2025	✗	vector production
GENE EDITING	**Casgevy™**	Vertex Pharmaceuticals	**beta thalassaemia and SCD**	February 2024	September 2025	
SOMATIC-CELL THERAPY MEDICINES	**Ebvallo®**	Pierre Fabre	**EBV+ PTLD**	December 2022	February 2025	
	Zemcelpro®	Cordex Biologics International Lim.	**haematological malignancies**	August 2025	November 2025*	
TISSUE-ENGINEERED MEDICINES	**Holoclar®**	Holostem	**limbal stem cell deficiency**	February 2015	February 2017	R&D, production, administration, vector production
	Spherox	CO.DON AG	**articular cartilage defects of the femoral condyle and the patella of the knee**	July 2017	November 2021*	

* *Therapies that have received marketing authorization from the Italian regulatory authority but are not reimbursed by the National Health Service, costs are covered entirely by patients.*

ATMP MANUFACTURING SITES IN ITALY: 13 ACADEMIC AND 7 PRIVATE FACILITIES

(LAST UPDATED ON 13 OCTOBER 2026)

PIEMONTE

- A UNIVERSITA' DEGLI STUDI DI TORINO
  CELL FACTORY MBC MOLECULAR BIOTECHNOLOGY CENTER
- A AZIENDA OSPEDALIERA CITTA DELLA SALUTE E DELLA SCIENZA DI TORINO
  CELL FACTORY OSPEDALE INFANTILE INFANTILE REGINA MARGHERITA

LOMBARDIA

- C AGC BIOLOGICS
- A AZIENDA OSPEDALIERA PAPA GIOVANNI XXIII
- A AZIENDA OSPEDALIERA S. GERARDO DI MONZA - VERRI
- A FONDAZIONE IRCCS POLICLINICO SAN MATTEO
- A FONDAZIONE IRCSS CA' GRANDA - OSPEDALE MAGGIORE POLICLINICO
- A ISTITUTO NEUROLOGICO CARLO BESTA
- C PATHEON

VENETO

- A FONDAZIONE BANCA DEGLI OCCHI DEL VENETO ONLUS
- A OSPEDALE SAN BORTOLO DI VICENZA

EMILIA ROMAGNA

- C EVOTEC
- C HOLOSTEM TERAPIE AVANZATE S.R.L.
- A ISTITUTO SCIENTIFICO ROMAGNOLO PER LO STUDIO E LA CURA DEI TUMORI

UMBRIA

- A AZIENDA OSPEDALIERA S. MARIA DI TERNI

LAZIO

- C ADVAXIA
- A OSPEDALE PEDIATRICO BAMBINO GESÙ
- C REITHERA

PUGLIA

- A UNITÀ PRODUTTIVA PER TERAPIE AVANZATE (UPTA)
  IRCCS FONDAZIONE "CASA SOLLIEVO DELLA SOFFERENZA"

CAMPANIA

- C INNOVAVECTOR



KEY/LEGEND

- **A** academia
- **C** company
- somatic cell therapy
- cell based gene therapy
- viral vector
- gene therapy (other)
- viral vector (starting material)
- tissue engineered products
- commercial products

DISCUSSION

Taken together, the findings present an ATMP ecosystem with a strong manufacturing base and a productive public research network, but with limited capacity to convert these assets into late-stage development and commercial growth. Italy plays a prominent role in the development and manufacturing of Advanced Therapy Medicinal Products (ATMPs), from early-stage development to commercial production. Five commercially approved advanced therapies are currently manufactured in Italy - four of them were also developed in our country -, indicating the presence of an established and resilient manufacturing base. This industrial capacity is complemented by a network of more than a dozen AIFA-authorized academic and hospital-based GMP facilities, which supports early-stage development and contributes to Italy's translational potential. Despite these strengths and Italy's record in translational research, the national ecosystem shows a marked imbalance. Scientific output and non-profit manufacturing capacity have not been matched by comparable late-stage clinical and industrial development, reflecting the so-called European european paradox. A limited presence of private biotechnology companies and start-ups appears to be a central component of this gap. This translational bottleneck is compounded by the absence of a coordinated support network for growth. More structured national and regional support is needed, together with greater participation by venture capital and business angel investors able to finance the transition from early development to clinical validation, scale-up, and commercialization.

CONCLUSION

- Closing these structural gaps requires stronger and more systematic collaboration between non-profit research organizations and the pharmaceutical industry, supported by a financial environment capable of attracting private investment throughout the scale-up phase.
- Alignment with strategic initiatives such as the proposed European Biotech Act will be important for Italy to remain connected to broader european ambitions and to create conditions that encourage greater commercial participation across the ATMP ecosystem.
- The strategic collaboration between NOTA and OTA can provide a platform for this transition by linking complementary capabilities: NOTA's focus on manufacturing innovation and regulatory harmonization, and OTA's expertise in policy advocacy and information outreach. Together, these activities can help connect scientific and manufacturing strengths with the policy and stakeholder engagement needed for ecosystem growth.
- Ultimately, sustained collaboration among academia, industry, public institutions, and financial stakeholders is needed to expand national manufacturing capacity and support the full development pathway, from early research to clinical translation and commercialization. Strengthening this continuum will help ensure that future ATMPs can be developed, manufactured, and made accessible to patients in Italy.

OSSERVATORIO TERAPIE AVANZATE (OTA)

OSSERVATORIO TERAPIE AVANZATE

Founded in 2019, Osservatorio Terapie Avanzate (OTA) is Italy's leading information hub dedicated to Advanced Therapy Medicinal Products, connecting researchers, clinicians, industry, institutions, and patients through rigorous science journalism and the support of a high standing international Scientific Committee.

Our impact & initiatives:

- information & outreach: creating and disseminating scientific articles, in-depth analyses, infographics, ebooks, vademecums, podcasts, and public events to build a stronger culture of ATMPs across Italy.
- media & editorial services: delivering customized contents, interviews, press office and release management, video coverage, and social media amplification for the sector.
- policy & governance: acting as an independent think tank to address regulatory, organizational, economic, and ethical challenges, producing position papers and white papers, and leading multidisciplinary advisory boards to support equitable access and effective governance.

info@osservatorioterapieavanzate.it

NETWORK OFFICINE TERAPIE AVANZATE APS

NOTA
NETWORK OFFICINE TERAPIE AVANZATE APS

Founded in 2022, NOTA-aps is the Italian network connecting organizations and professionals across the Advanced Therapy Medicinal Products ecosystem, from preclinical and clinical research to manufacturing, supply chains, clinical application, and regulation.

Our impact & initiatives:

- accelerating Innovation: fostering collaboration and knowledge exchange to address ATMP manufacturing challenges and improve patient outcomes.
- regulatory harmonization: working alongside national and international authorities to align and harmonize industry guidelines.
- ecosystem support: driving professional networking, continuing education, and sector-wide initiatives to support the people shaping the future of advanced therapies.

comunicazione@nota-aps.org