Review And Horizon Scanning Of Advanced Therapy Medicinal Products In Slovenia: Registration, Reimbursement And Future Pipeline

HPR176

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Background

Advanced therapy medicinal products (ATMPs) represent the most innovative and technologically advanced form of treatment, categorized into **gene therapy**, **somatic-cell therapy**, **tissue-engineered products**, and **combined ATMPs**.

Objective

To map the landscape of ATMPs in Slovenia and to perform horizon scanning of emerging ATMPs (next 2 years), identifying gaps in policy and health technology assessment readiness.

Methods

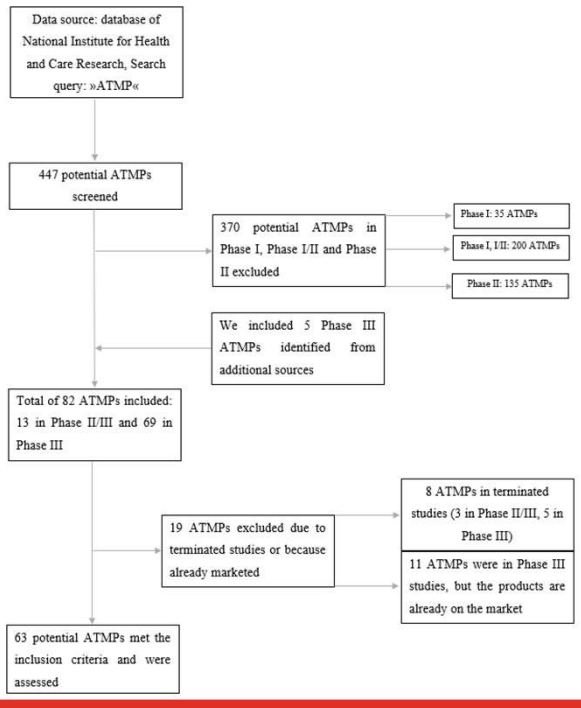
Time frame: until October 2024

Phase 1:

- Identification of all ATMPs with European Committee authorization
- Cross-check of identified ATMPs for Slovenian marketing authorizations and reimbursement status

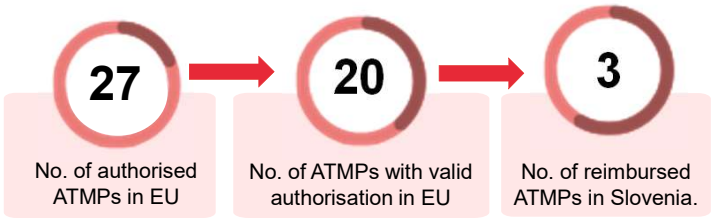
Phase 2:

- Horizon scanning of emerging ATMPs
- Inclusion criteria: ATMPs in II./III. and III. phase of clinical trials



Results

1. ATMPs landscape in EU and Slovenia



ATMPs categorised by type in the EU:

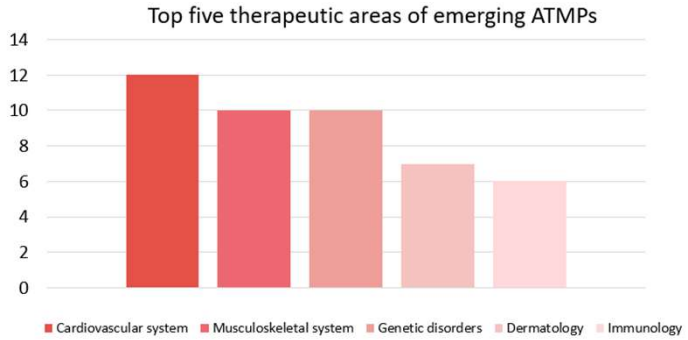
- Gene therapy: 80%
- Somatic-cell therapy: 10%
- Tissue-engineered products: 10%

Authorised ATMPs registered for rare diseases: **80 %**

PROPRIETARY NAME OF MEDICINE	ACTIVE SUBSTANCE	ATMP CATEGORY	THERAPEUTIC AREA	MEDICINE PRICE IN SLOVENIA	TOTAL VALUE OF PRESCRIPTIONS IN 2024
Alofisel® *	Darvadstrocel	Somatic-cell therapy	Diseases of the digestive system and metabolic disorders	51 327 €	204 472 €
Kymriah®	Tisagenlecleucel	Gene therapy	Neoplasms (haematologic malignancies)	239 027 €	1 281 150 €
Zolgensma®	Onasemnogene abeparvovec	Gene therapy	Diseases of the musculoskeletal system and connective tissue	1 385 027 €	0 €

*= medicine lost marketing authorisation in Dec 2024

2. Emerging ATMPs, N=63



Emerging ATMPs categorised by type:

- Gene therapy: 41 %
- Somatic-cell therapy: 27 %
- Tissue-engineered products: 29 %
- Combined ATMPs: 3 %

Emerging ATMPs for rare diseases: **46 %**

Conclusion

The number of approved ATMPs in the EU is low, and several of them lost marketing authorisation. In Slovenia, only three ATMPs were reimbursed in 2024, which is consistent with the national needs in the area of rare diseases. Among newly emerging therapies, somatic cell and tissue-engineered products are becoming more prominent alongside gene therapies, which have dominated so far. A shift is also observed in the proportion of therapies targeting rare diseases.