INTRODUCTION:

- According to EMA (European Medicines Agency) definition¹, Advanced therapy medicinal products (ATMPs) are medicines for human use that are based on genes, tissues or cells. They offer groundbreaking new opportunities for the treatment of disease and injury.

- All advanced therapy medicines are authorized centrally via the EMA.

- However, due to their specificities in terms of manufacturing and patient evaluation, the market access for ATMPs across European countries is complex and varies hugely depending on the country.

OBJECTIVE:

- The aim of this poster is to compare the market access for ATMPs approved by the EMA since 2015 across the EUS countries focusing on reimbursement trends.

METHODS:

- 11 innovative ATMPs were identified with an EMA market authorization (including Zolgensma® which was withdrawn in 2019). Since the similar analysis we done in March 2019, only one ATMP has been approved: Zolgensma®.

- A targeted research on official EUS reimbursement / HTA agencies websites were executed in June 2020 in order to describe the current market access situation for each ATMP.

RESULTS:

- The reimbursement and pricing vary between countries: the table below describes the current market access situation of each ATMP in the EUS.

- For France, 8/11 ATMPs were assessed for reimbursement by the transparency committee: Six had a positive reimbursement decision (Holoclar®, Alofisel®, Kyriphan®; Yescarta®; LYXTANA® and Zynteglo®). Two had a negative reimbursement decision (Zolgensma®, Spherobox®).

- Compared to 2019 analysis, two more were assessed: Zytegl® was recognized as innovative (moderate III added medical benefit) and Spherox® had a negative reimbursement decision.

- For Germany, 7 ATMPs were assessed within the AMNOG process (Ilyngig®, Zolgensma®, Alofisel®, Kyriphan®, Yescarta®, LXUTANA®, Zytegl®) and 1/11 (Zolgensma®) is expected by the end of 2020.

- Kyriphan® was reassessed with no change of the added benefit.

- Compared to 2019 analysis, two more therapies were assessed by G-BA (Zytegl®) which obtain a non quantifiable added benefit and LXUTANA® which obtain a hint for a considerable added benefit.

- Holoclar® was taken as a procedure and it can be reimbursed through the DRG-System.

- Kyriphan® was the 1st ever therapy on which a pay-for-performance deal was agreed.

- Zolgensma®, has follow the new rule of the “Anwendungsbegleitende Datenberatung” introduced at the end of 2019. The required Routine Practice Data Collection according §§35a Par. 3b SGB V binds the manufacturer to set up a patient registry and to submit results yearly within a new AMNOG Dossier to the G-BA.

- For Spain, 3/11 drugs (vs 2/10 in 2019) are reimbursed (Kyriphan®, Yescarta® and Alofisel®), all of them included in Valtermed with a payment by results agreement. The therapeutic positioning report (RPT) of Alofisel® included restrictions in the patient population.

- Strimvelis®, Spherobox® and Zytegl® have not requested a national code. Therefore, they have not been evaluated in Spain.

- Holoclar®, Ilyngig® and Zolgensma® have a negative reimbursement decision. In spite of not being reimbursed, Holoclar® is commercialized.

- LXUTANA® and Zolgensma® are currently under evaluation. National codes for LXUTANA® and Zolgensma® were issued, respectively, in April 2019 and in July 2020.

- For the United Kingdom, 7/11 drugs (vs 5/10 in 2019) are reimbursed to date: only one of these, Strimvelis®, was reimbursed at its full list price.

- Spherobox® was reimbursed via NICE’s STA with some restrictions.

- The two CAR-Ts were reimbursed via NHS England’s Cancer Drugs Fund.

- Holoclar®, Ilyngig® and LXUTANA® were recommended using a simple discount Patient Access Scheme.

- Kyriphan® and Zolgensma® are currently being appraised by NICE.

- Alofisel® was not recommended by NICE.

CONCLUSIONS:

- Most ATMPs are granted patient access in EUS even though HTA bodies have imposed monitoring requirements to ensure the value reflects the price.

- Access to ATMPs varies across countries. Germany and the UK are the countries with the highest number of reimbursed ATMPs whereas Spain is the country with the lowest number.

- At this time, Kyriphan® and Yescarta® are the only ATMPs reimbursed in the all EUS countries.

REFERENCES:


- Haufe Autoritäten der Welt. Available at [http://www.hau-fe.de]


- Informe de posicionamiento terapéutico. Available at [https://www.aferi.gob.es/medicamento-de-uso-humano/Informes-de-posicionamiento-terapeutico]


- Gemeinsamer Bundesausschuß (G-BA). 2020. Verfügung über die Medizinprodukteverordnung §§ 25a bis 31a SGB V. Available at [https://www.g-ba.de]


- Haufe Autoritäten der Welt. Available at [http://www.hau-fe.de]


- Informe de posicionamiento terapéutico. Available at [https://www.aferi.gob.es/medicamento-de-uso-humano/Informes-de-posicionamiento-terapeutico]


- Gemeinsamer Bundesausschuß (G-BA). 2020. Verfügung über die Medizinprodukteverordnung §§ 25a bis 31a SGB V. Available at [https://www.g-ba.de]