TO REVIEW THE PRMA LANDSCAPE FOR CELL AND GENE THERAPIES AND ATMPs APPROVED IN THE US AND EUROPE

OBJECTIVE

To examine the pricing, reimbursement and market access (PRMA) landscape for cell and gene therapies and ATMPs approved in the US and Europe

METHODS

• Reviewed all cell/gene therapies and ATMPs approved as of November 2021 and available in the US, EU (France, Germany, Italy, Spain) and the US as of April 2022
• Also noted that products have been withdrawn
• Analyzed HTA, pricing, regulatory and market access status and time to market
• Data gathered from EMA, national Health Technology Assessment (HTA) agencies and Pricing and Reimbursement (P&R) bodies
• Sources for launch dates and HTA information provided in Table 1 below

RESULTS

• Market access status of cell and gene therapies, ATMPs varies considerably across the six countries (Figure 2)
  - US: 100% of the cell and gene therapies are reimbursed under medical benefit
  - France: 80% only 50% of therapies have completed negotiations and received positive reimbursement recommendations, patients do currently have access to an additional 35% following negotiations via the early access pathway (overall 83%)
  - Germany: 57% of ATMPs are reimbursed following completion of the AMNOG procedure
    • At additional 25% of therapies are currently undergoing negotiations and patients have access given reimbursement is immediate
    • Germany has the lowest (6%) of therapies reimbursed, all with patient registries and several with PBPs (P&R agreements)
  - UK: 83% of ATMPs are currently recommended
    • Interestingly only three have been assessed by highly specialized (HTS) technologies pathway, which severely specifically designed for them
  - EU: 83% of ATMPs are currently recommended for reimbursement by NICE
  - US: 82% of therapies are either currently reimbursed or have been withdrawn from these markets
  - Only 2 therapies that are no longer marketed are available in the US

• Data above does NOT include ATMPs that have been withdrawn from these markets

Conclusions

• Cell/gene therapy and ATMP markets are still developing as evidenced by the fragmented regulatory and market access landscape
• Regulatory approval generally tends to be consistent across the US and EU, however this is not the case for cell/gene therapies and ATMPs
• For manufacturers, this is not a traditional market as evidenced by the heterogeneity in regulatory environment, HTA, acceptable prices and market access pathway(s)
• Market access remains challenging particularly in Europe where 7 therapies have been withdrawn for clinical/commercial reasons
• Within Europe, there is a huge variation in access with UK and Germany leading the way with largest number of reimbursed therapies and Spain lagging behind, although this has improved over the past year
• Type of therapy also impacts access with CAR-Ts and gene therapies for rare pediatric conditions more likely to be reimbursed, at least in Europe, relative to ‘tissue engineered therapies’
• Visible ex-factory prices of recently introduced cell and gene therapies are still setting new highs, however, contracting in the US has been rapid, due to confidential discounts / managed access agreements (in Europe) often reduce net prices
• In general, gross to net differences in Europe are becoming increasingly important, with overall discounts for reimbursed cell and gene therapies ranging from 10% to 40%
• Gross to net differences may grow in future, especially within more “competitive” categories (e.g. CAR-Ts) which are also a consequence of gradual expansion into earlier lines of treatment / broader indications
• Innovative approaches to market access will continue to evolve, as more cell/gene therapies and ATMPs are launched, bringing important health benefits, but also with substantial potential impact on pharmaceutical budgets