Introduction

Advanced Therapy Medicinal Products (ATMPs) include cell therapies, gene therapies, tissue and tissue derived products. These products often require long and costly development cycles. Payers often struggle with the HTA process of ATMPs due to their high costs and unique characteristics. Moreover, after launch, challenges relating to long-term benefits and high upfront (or mid-term) costs lead to clinical uncertainty over longer periods. These payers are often faced with decisions not to fund ATMPs or to fund them with limited patient access at the time of launch, thus not informing all patients the system needs to achieve sustainable health systems.

The HTA process may need to be revised to provide the opportunity for early engagement with manufacturers developing high-priority ATMPs.

Objectives

The research aims to understand whether the HTA process of ATMPs is adequate to assess the clinical value of ATMPs and achieve sustainable reimbursement. The research investigated the HTA process and patient preferences for managing the access and reimbursement of ATMPs. The research also aimed to discuss sustainable access models for ATMPs.

Methods

The research involved an advisory meeting with EU and US payers and industry executives in July 2020. The research also involved interviews with industry stakeholders from the UK and Germany. Semi-structured interviews were conducted with 18 payers, including HTA, budget setting and sickness funds from the EU5 and US in September 2020.

Results

The HTA process may need to be revised to provide the opportunity for early engagement with manufacturers developing high-priority ATMPs.

The HTA process may need to be revised to provide the opportunity for early engagement with manufacturers developing high-priority ATMPs.

Conclusions and recommendations

For ATMPs with a short-to-mid-term duration of effect, payers consider conditional reimbursement and pay for performance as the preferred access model. For ATMPs with a long-term duration of effect, there is increased payer interest in performance-based payment models and annuity payments.

The HTA process may need to be revised to provide the opportunity for early engagement with manufacturers developing high-priority ATMPs.

The HTA process may need to be revised to provide the opportunity for early engagement with manufacturers developing high-priority ATMPs.

Recommendations for inter-industry collaboration

An advisory meeting including European and US payers and industry executives was held in July 2020 to discuss sustainable access models for ATMPs. Meeting participants aligned on the need for inter-industry collaboration to agree a common framework to address the challenges of developing ATMPs, and to discuss sustainable access models for ATMPs. Meeting participants recommended to discuss sustainable access models for ATMPs, and to discuss sustainable access models for ATMPs.

Payers and industry stakeholders recognise the value of international collaboration between clinical societies, governments and industry to develop a framework of global emerging needs and clinical priorities for ATMPs.

For industry, this should reduce uncertainty and lead therefore the value of investment in new ATMPs for specific indications. Collaboration will inform a common understanding of the different levels of uncertainty for ATMPs at launch and models to reflect ongoing data generation in pricing and access decisions. This will also reduce uncertainty to develop a variety of international specific payment models for ATMPs to facilitate sustainable equitable access for payers. For payers HTA bodies, this should enable a more objective approach to value assessment and to develop a wide array of manufacturing high value-added ATMPs, ensuring active involvement in the pre- and post-launch strategy generation strategy.

Recommendaclions for inter-industry collaboration

An advisory meeting including European and US payers and industry executives was held in July 2020 to discuss sustainable access models for ATMPs. Meeting participants aligned on the need for inter-industry collaboration to agree a common framework to address the challenges of developing ATMPs, and to discuss sustainable access models for ATMPs. Meeting participants recommended to discuss sustainable access models for ATMPs, and to discuss sustainable access models for ATMPs.

Payers and industry stakeholders recognise the value of international collaboration between clinical societies, governments and industry to develop a framework of global emerging needs and clinical priorities for ATMPs.

For industry, this should reduce uncertainty and lead therefore the value of investment in new ATMPs for specific indications. Collaboration will inform a common understanding of the different levels of uncertainty for ATMPs at launch and models to reflect ongoing data generation in pricing and access decisions. This will also reduce uncertainty to develop a variety of international specific payment models for ATMPs to facilitate sustainable equitable access for payers. For payers HTA bodies, this should enable a more objective approach to value assessment and to develop a wide array of manufacturing high value-added ATMPs, ensuring active involvement in the pre- and post-launch strategy generation strategy.

Recommendaclions for inter-industry collaboration

An advisory meeting including European and US payers and industry executives was held in July 2020 to discuss sustainable access models for ATMPs. Meeting participants aligned on the need for inter-industry collaboration to agree a common framework to address the challenges of developing ATMPs, and to discuss sustainable access models for ATMPs. Meeting participants recommended to discuss sustainable access models for ATMPs, and to discuss sustainable access models for ATMPs.

Payers and industry stakeholders recognise the value of international collaboration between clinical societies, governments and industry to develop a framework of global emerging needs and clinical priorities for ATMPs.

For industry, this should reduce uncertainty and lead therefore the value of investment in new ATMPs for specific indications. Collaboration will inform a common understanding of the different levels of uncertainty for ATMPs at launch and models to reflect ongoing data generation in pricing and access decisions. This will also reduce uncertainty to develop a variety of international specific payment models for ATMPs to facilitate sustainable equitable access for payers. For payers HTA bodies, this should enable a more objective approach to value assessment and to develop a wide array of manufacturing high value-added ATMPs, ensuring active involvement in the pre- and post-launch strategy generation strategy.

Recommendaclions for inter-industry collaboration

An advisory meeting including European and US payers and industry executives was held in July 2020 to discuss sustainable access models for ATMPs. Meeting participants aligned on the need for inter-industry collaboration to agree a common framework to address the challenges of developing ATMPs, and to discuss sustainable access models for ATMPs. Meeting participants recommended to discuss sustainable access models for ATMPs, and to discuss sustainable access models for ATMPs.

Payers and industry stakeholders recognise the value of international collaboration between clinical societies, governments and industry to develop a framework of global emerging needs and clinical priorities for ATMPs.

For industry, this should reduce uncertainty and lead therefore the value of investment in new ATMPs for specific indications. Collaboration will inform a common understanding of the different levels of uncertainty for ATMPs at launch and models to reflect ongoing data generation in pricing and access decisions. This will also reduce uncertainty to develop a variety of international specific payment models for ATMPs to facilitate sustainable equitable access for payers. For payers HTA bodies, this should enable a more objective approach to value assessment and to develop a wide array of manufacturing high value-added ATMPs, ensuring active involvement in the pre- and post-launch strategy generation strategy.

An advisory meeting including European and US payers and industry executives was held in July 2020 to discuss sustainable access models for ATMPs. Meeting participants aligned on the need for inter-industry collaboration to agree a common framework to address the challenges of developing ATMPs, and to discuss sustainable access models for ATMPs. Meeting participants recommended to discuss sustainable access models for ATMPs, and to discuss sustainable access models for ATMPs.

Payers and industry stakeholders recognise the value of international collaboration between clinical societies, governments and industry to develop a framework of global emerging needs and clinical priorities for ATMPs.

For industry, this should reduce uncertainty and lead therefore the value of investment in new ATMPs for specific indications. Collaboration will inform a common understanding of the different levels of uncertainty for ATMPs at launch and models to reflect ongoing data generation in pricing and access decisions. This will also reduce uncertainty to develop a variety of international specific payment models for ATMPs to facilitate sustainable equitable access for payers. For payers HTA bodies, this should enable a more objective approach to value assessment and to develop a wide array of manufacturing high value-added ATMPs, ensuring active involvement in the pre- and post-launch strategy generation strategy.

For ATMPs, inter-industry collaboration is required to address the challenges of ATMP review.

Conclusion

Since there have been five withdrawals of ATMPs from the European market since 2014, principally due to the failure to show clinical benefit (e.g., Zolgensma), there is a need to develop a transparent framework to address the challenges of developing ATMPs, and to discuss sustainable access models for ATMPs. Meeting participants recommended to discuss sustainable access models for ATMPs, and to discuss sustainable access models for ATMPs.

Payers and industry stakeholders recognise the value of international collaboration between clinical societies, governments and industry to develop a framework of global emerging needs and clinical priorities for ATMPs.

For industry, this should reduce uncertainty and lead therefore the value of investment in new ATMPs for specific indications. Collaboration will inform a common understanding of the different levels of uncertainty for ATMPs at launch and models to reflect ongoing data generation in pricing and access decisions. This will also reduce uncertainty to develop a variety of international specific payment models for ATMPs to facilitate sustainable equitable access for payers. For payers HTA bodies, this should enable a more objective approach to value assessment and to develop a wide array of manufacturing high value-added ATMPs, ensuring active involvement in the pre- and post-launch strategy generation strategy.

For ATMPs, inter-industry collaboration is required to address the challenges of ATMP review.

Conclusion

Since there have been five withdrawals of ATMPs from the European market since 2014, principally due to the failure to show clinical benefit (e.g., Zolgensma), there is a need to develop a transparent framework to address the challenges of developing ATMPs, and to discuss sustainable access models for ATMPs. Meeting participants recommended to discuss sustainable access models for ATMPs, and to discuss sustainable access models for ATMPs.

Payers and industry stakeholders recognise the value of international collaboration between clinical societies, governments and industry to develop a framework of global emerging needs and clinical priorities for ATMPs.

For industry, this should reduce uncertainty and lead therefore the value of investment in new ATMPs for specific indications. Collaboration will inform a common understanding of the different levels of uncertainty for ATMPs at launch and models to reflect ongoing data generation in pricing and access decisions. This will also reduce uncertainty to develop a variety of international specific payment models for ATMPs to facilitate sustainable equitable access for payers. For payers HTA bodies, this should enable a more objective approach to value assessment and to develop a wide array of manufacturing high value-added ATMPs, ensuring active involvement in the pre- and post-launch strategy generation strategy.