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European benchmark

ATMP market entry from an HTA perspective

Region Skåne

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Executive summary

HTA bodies in Europe have, in general, not established specific mechanisms to adequately capture the full benefits of ATMPs

Region Skåne engaged Monocl Strategy & Communication (MSC) in a project to increase the knowledge on what extent Health Technology Assessments (HTAs) have on market access of Advanced Therapeutic Medicinal Products (ATMPs) and what payment models are available in selected European countries. The analysis covers Norway, Finland, Denmark, Germany, Netherlands, United Kingdom, France, Spain and Italy.

MSC made a background research and data analysis of the countries' HTA systems in relation to ATMPs using public and proprietary sources/databases.

- All the selected European countries have their own HTA bodies, which all seek value-for-money from medicinal products. However, the individual countries HTA bodies have different priorities and methods which influences the result. In some countries the are more then one HTA body and in some countries the are several regional HTA bodies working somewhat independently for examples in Italy and Spain.
- None of the HTA bodies have specialized committees with dedicated expertise on ATMPs. Due to this and the lack of established mechanisms to capture the full benefit of ATMPs in HTAs some experts have expressed that there are risk that these therapies might not reach patients in need in a timely manner.
- Most of the approved ATMPs have undergone HTAs evaluations. So far this has been restricted to a small number of products largely targeting hemato-oncological indications. It will take some time before a significant amount of data is available to inform reimbursement strategies in Europe based on these HTA evaluations and approvals. At the same time, the ongoing debate about whether current HTA methods are best suited for the appraisal of ATMPs remains unresolved.
- There are payment issues related to ATMPs, but there are new payment models like conditional reimbursement, pay-for-performance and annuity-based payment models emerging addressing these issues. These are far from established and in full use yet and they are still under development and only used in some cases. We will most likely see more results and publications from these initiatives coming years. Some countries has been more progressive in trying out new pricing and paying models e.g. in Italy and Germany. But discussion on how to address the high cost of ATMP is ongoing in most countries. The countries have also different susceptibility to adapting new forms of evidence (e.g. Real World Evidence) in their assessments which consequently creates a discrepancy in reimbursements between the countries.
- The European Commission adopted a new proposal in 2018 regarding HTA. With the aim HTA to strengthen EU-level cooperation among Member States for assessing health technologies. According to the Commission, it would not only make innovative health tools reach patients faster, but also boost innovation and improve competitiveness of the European healthcare sector. cooperation.

ATMP market entry from an HTA perspective

Advanced Therapy Medicinal Products in Europe

Advanced therapeutic medicinal products (ATMPs) are medicines for human use based on genes, cells or tissue engineering

- Cell and gene therapies are designed to have longer-lasting effects than most traditional medicines. Many target the underlying biology of disease, rather than its symptoms. This means they can dramatically improve health outcomes and potentially **offer a cure**. ATMPs can also address complex diseases for which there are no effective conventional treatments.
- Regulators have established dedicated pathways and expert committees to help ensure appropriate, expedited marketing authorization of ATMPs. The European Parliament introduced the concept of ATMPs in 2007, triggering the creation of the **European Medicines Agency's Committee for Advanced Therapies (CAT)**. In the US, FDA has created **Center for Biologics Evaluation and Research (CBER) dedicated for ATMPs**.

ATMPs can be classified into three main categories (EUs CAT):

- **Gene therapy medicines (GTMP)**: these contain genes that lead to a therapeutic, prophylactic or diagnostic effect. They work by inserting 'recombinant' genes into the body, usually to treat a variety of diseases, including genetic disorders, cancer or long-term diseases. A recombinant gene is a stretch of DNA that is created in the laboratory, bringing together DNA from different sources;
- **Somatic-cell therapy medicines (sCTMP)**: these contain cells or tissues that have been manipulated to change their biological characteristics or cells or tissues not intended to be used for the same essential functions in the body. They can be used to cure, diagnose or prevent diseases. Most often just called cell therapy.
- **Tissue-engineered medicines (TEP)**: these contain cells or tissues that have been modified so they can be used to repair, regenerate or replace human tissue

ATMPs in EUROPE,
end of Q3 2019

Phase	Number
Marketed	10
Phase 3	177
Phase 2	259
Phase 1	61

There are ten approved ATMPs in Europe with a centralized license

The EMA has a centralized marketing authorization process and one license is valid in the entire European Union. From start to finish, the authorization procedure takes 210 days with a stop-clock after the primary evaluation.

New therapies are reviewed by different committees in the EMA where the Committee for Advanced Therapies (CAT) drafts an opinion and the Committee for Medicinal Products for Human Use (CHMP) adopts a final opinion on a potential market authorization.

MARKETING AUTHORIZATION OF ATMPs

Pre-submission

The primary evaluation last between day 0-120.

Secondary evaluation

The secondary evaluation last between day 121-210.
An opinion is created by an EMA committee and the decision is made by the European commission.

Approval

After the approval, post authorization activities begin.

10 ATMP approved in Europe, 17 in the USA

ATMPs APPROVED IN EUROPE

	Brand name	MA date			Status
Gene therapies	*Glybera*	*Oct 2010*	*Hyperlipoproteinemia type I*	uniQure	*Withdrawn*
	Imlygic	Dec 2015	Melanoma	Amgen	Authorized
	Strimvelis	May 2016	SCID	Orchard Therapeutics	Authorized
	Kymriah	Sep 2018	Relapsed or refractory DLBCL & B cell precursor ALL	Novartis	Authorized
	Yescarta	Sep 2018	Relapsed or refractory DLBCL and PMBCL	Gilead Company	Authorized
	Luxturna	Nov 2018	Retinal disease	Spark Therapeutics	Authorized
	Zynteglo	Jun 2019	Beta thalassaemia in patients	BlueBird	Authorized
Cell therapies	*Provenge*	*Sep 2013*	*Prostatic neoplasms*	Dendreon Pharmaceuticals	*Withdrawn*
	Zalmoxis	Aug 2016	HSCT adjunctive treatment	MolMed	Authorized
	Alofisel	Mar 2018	Rectal fistula	Takeda	Authorized
	Chondrocelect	*Oct 2009*	*Cartilage diseases*	TiGenix	*Withdrawn*
	Maci	*Jun 2013*	*Fractures, cartilage*	Vericel	*Withdrawn*
Tissue-based therapies	Holoclar	Feb 2015	Corneal diseases	Chiesi Farmaceutici	Authorized
	Spherox	Jul 2017	Cartilage diseases	CO.DON	Authorized

- There are 10 ATMP approved in Europe by EMA, compared to 17 in the US.
- Four products have been withdrawn from the market.
- Latest to get approved was Bluebird gene therapy Zynteglo.

The centralized committee CAT in EU covers scientific areas relevant to advanced therapies

The Committee for Advanced Therapies (CAT) is the European Medicines Agency's (EMA) committee responsible for assessing the quality, safety and efficacy of advanced therapy medicinal products (ATMPs) and following scientific developments in the field.

EUROPEAN MEDICINES AGENCY
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ACTIVITIES PERFROMED BY CAT

- Participates in certifying quality and non-clinical data for SMEs developing ATMPs and in providing scientific recommendations on classifications of ATMPs
- Contributes to scientific advice, in cooperation with the Scientific Advice Working Party (SAWP)
- Takes part in any procedure delivering advice on the conduct of efficacy follow-up, pharmacovigilance or risk-management systems for ATMPs
- Advises the CHMP on any medicinal product that may require expertise in ATMPs for the evaluation of its quality, safety or efficacy
- Assists scientifically in developing any documents relating to the objectives of the Regulation on ATMPs
- Provides scientific expertise and advice for any Community initiative related to the development of innovative medicines and therapies that requires expertise on ATMPs
- Supports the work programs of the CHMP working parties

ATMP market entry from an HTA perspective

Health Technology Assessment in Europe

Health technology assessments are used to evaluate the properties and effect of health technologies

Health technology assessment (HTA)

- **HTA** is the systematic evaluation of the properties and effects of a health technology, addressing the **direct and intended effects** of this technology, as well as its **indirect and unintended consequences**, and aimed mainly at informing decision making regarding health technologies. HTA is conducted by interdisciplinary groups that use explicit analytical frameworks drawing on a variety of methods.
- **All HTA bodies seek value-for-money from medicinal products. However, how this is done by the individual HTA bodies in EU nations differs in priorities and methods.** The degree to which the HTA bodies can influence negotiated prices are linked to their nation's specific health system funding model and the weighting of economic/budget impact versus broader clinical societal impact.
- Some nations' HTA bodies are more willing than others to accept new kinds of evidence beyond traditional randomized controlled trials and to consider economic models that involve extrapolating longer-term benefit from limited existing data.

A **health technology** is defined as an intervention that may be used to promote health, to prevent, diagnose or treat acute or chronic disease, or for rehabilitation.

Health technologies include pharmaceuticals, devices, procedures and organizational systems used in health care.

HTA bodies have not yet established mechanisms to capture the benefits of ATMPs

- ATMPs are associated with high up-front cost compared to traditional treatments, caused in part by complex processes for manufacturing and administration, but primarily due to the long-term value to patients, society, and health systems and administration provided by a one-time treatment.
- Little of the value of ATMPs, which may come over time in terms of savings on treatments and procedures that are no longer necessary and in terms of quality of life and productivity, can be adequately captured in current value-assessment frameworks.
- Because of the nature of ATMPs, many of them may not have developed the comparative evidence versus standard of care at time of launch which HTA bodies traditionally require. **The HTA bodies do not yet have a specialized committee with dedicated expertise in ATMPs like CAT, which creates an expertise gap within HTA bodies.**
- **Most payers and HTA bodies have not established specific mechanisms to adequately capture the full benefits of ATMPs.** Consequently, there are many systematic barriers that may hinder ATMPs from reaching patients in need in a timely manner.
- Specific pathways that help ensure ATMP treatments to reach those in need as quickly and safely as possible need to be established. This requires new approaches to measuring value for ATMPs, akin to the innovative and potentially transformative impact that ATMPs can give.
- These approaches also have to offer payers affordable, risk-mitigated means of funding ATMPs, with evidence-based reassurance that healthcare systems are getting value for money and with the commitment to the generation of long-term evidence.

Individual HTA bodies in the EU have different priorities and methods in their assessments

Country/HTA Agency	Method driving HTA recommendations	HTA perspective (economic analysis)	Value judgement			Degree of influence on price/rebate
			Clinical benefit	Cost-effectiveness analysis	Budget impact	
France / HAS (TC, CEESP)	Mixed model: usually clinical, in some cases health economic	Payer (collective perspective)	High	High*/1	High 1	Moderate (benefit tier determines reimbursement level)
Germany / IQWIG (consultative), G-BA	Clinical model (G-BA)	Payer (only drug budget impact)	High	Low 1	Low	High (decision influences pricing negs.)
Italy / AIFA, regions	Mixed model: clinical for national decisions, sometimes health economic at regional level	Payer	High	Low	High/ Moderate	High — AIFA and regions negotiate prices
Spain / SGCMPS, regions	Mixed model: clinical for national decisions, sometimes health economic at regional level	Payer	High	Low	High	High — central and regional negotiations; ref. pricing
UK / NICE (England), SMC (Scotland)	Health economic model2	National health system and personal social services	High	High	Low	Moderate-High2

The table is adapted from Alliance for Regenerative Medicine's (ARM) consensus report on "Recommendations for Timely Access to Advanced Therapy Medicinal Products in Europe" based on ARM's primary research.

*No formal threshold; 1) only in certain cases/products; 2) clinical aspects are taken into consideration during the process and fed into the HE model.

HAS: Haute Authorité de Santé, IQWIG: Institute for Quality and Efficiency in Health Care, G-BA: Federal Joint Committee, AIFA: Italian Medicines Agency, SGCMPS: General Subdirectorate of Quality of Medicines and Health Products, NICE: National Institute for Health and Care Excellence, SMC: Scottish Medicines Consortium

Innovative payment models have been proposed, but challenges exist that halt the implementation

Reimbursement models

- There are several **new payment models, such as conditional reimbursement, pay-for- performance, and annuity-based payments recommended** under evaluation for ATMP.
- **Conditional reimbursement** is an agreement consisting of reimbursement linked to the collection of post-launch evidence, such as Real-World-Evidence (RWE).
  - After collecting prospective population-level evidence from a pre-specified study, the reimbursement is reassessed and there is a possibility to either expand or withdraw the coverage.
  - This coverage evidence development (CED) can be applied when novel medical technologies are promising, yet additional evidence is required to make an informed decision.
  - Increasingly considered as a useful policy instrument since it allows collection of evidence regarding effectiveness and cost-effectiveness of new technologies without delaying market access.
- **Several payment models that can be used together with conditional reimbursement have been proposed**. Description and challenges associated with them are presented to the right.

PAYMENT MODELS AND THEIR CHALLENGES

	Payment details	Challenges
Annuitized payments	Payments **spread over years to overcome affordability issues** and soften "sticker shock" regardless of performance.	One-time hit avoided, but long-term **budget impact remains unchanged**. Patient might **leave plan**.
Pay-for-performance	Payment only initiated if **predetermined goals are met** or rebate issued if goals are missed.	Need to define and **track long-term outcomes**.
Annuitized payment + Pay-for-performance	Similar to annuitized payments, **but payment only sent if treatment goals are met**.	Need to define and **track outcomes long-term**. Patient might **leave plan**.
Discount based on % paid up-front	Payment is spread over time, but payer receives a **discount based on initial payment** percentage.	Does not **protect from risk** of treatment failure.

ATMP market entry from an HTA perspective

National HTAs in ATMPs

Highlights from nation-specific HTA practices and ATMP market access

- Several of the analyzed countries, such as France, Spain and Italy, have more than one body conducting HTA. For example one body is responsible for pharmaceuticals and the other for medical devices. In some countries, there are additionally regional authorities that conduct HTA. Italy and Spain are two examples of this.
- HTA are used to give guidance for approval and/or reimbursement but how much they influence they might have on approval decision, labelling and price differs between the countries. In Norway, Denmark, Finland, UK, Spain and Italy the HTA bodies have a more advisory role whereas decision-makers in the Netherlands and Germany are bound to follow the guidance from HTA bodies.
- None of the HTA bodies analyzed in this study have put in place specialized with dedicated expertise of ATMPs in place.
- There are many different versions of HTA in use in the selected countries. And within a country there are different types of HTA being done. In Norway for example there HTAs formats; mini-HTA, STA (single technology assessment and full HTA.
- Several countries have started introducing more advanced payment and pricing models for advanced and very cost intensive therapies like ATMPs. For example, there are several versions of pay-for-success or result as an alternative to a more straight-forward reimbursement with a list price in use. Italy is one nation that stands out, but also Spain and Germany have introduced alternative payment models.
- The Finnish government introduced conditional reimbursement in 2017. It is risk sharing model for new drugs with limited study evidence and user experience.

ATMP market entry from an HTA perspective

Norwegian perspective

Norway - Overview

National HTA organization	The National System for Managed Introduction of New Health Technologies within the Specialist Health Service / *Nye Metoder*
Purposes of HTA	Clinical practice guidelines and protocols, planning and budgeting
How HTA is used in decision-making	Advisory, decision makers rely partly on the advice

HTA reviewed approved ATMP

Brand name	ATMP technology	HTA assessment of therapy	Type of HTA assessment	Payment method
Imlygic	GTMP	Uncertain whether Imlygic fulfils the conditions to be recommended for implementation.	Single technology assessment (STA)	No advanced payment method
Kymriah	GTMP	Estimated gain too uncertain. Additional follow-up data needed to evaluate and reduce the uncertainty.	Single technology assessment (STA)	No advanced payment method
Yescarta	GTMP	Estimated gain too uncertain. Additional follow-up data needed to evaluate and reduce the uncertainty.	Single technology assessment (STA)	No advanced payment method
Alofisel	sCTMP	Not cost-effective enough given the severity of patient group.	Single technology assessment (STA)	No advanced payment method

Norway – Pricing and HTA methodology

Pricing

- The Norwegian Medicines Agency is responsible for setting maximum prices on prescription-only-medicines. The agency also evaluates and decides whether a medicinal product should be publicly funded.
- Denmark, Norway and Iceland have started working together to negotiate prices on expensive pharmaceuticals.
- **We have not identified any new type of pricing model for approved the ATMPs.**

HTA

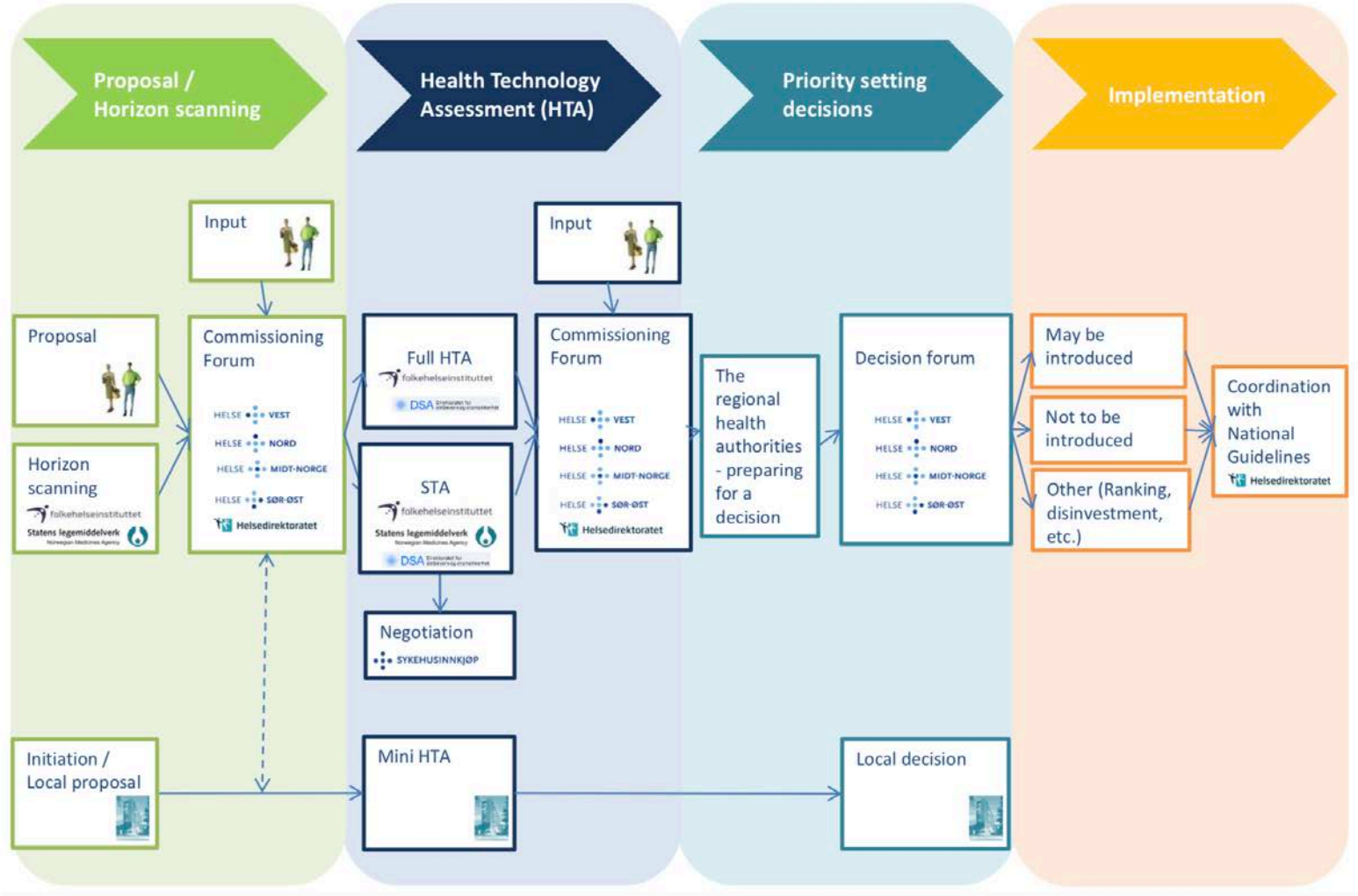
- Norway uses a health economic evaluation which takes a societal perspective with indirect costs of treatment and illness into account.
- There are three HTAs formats in Norway; **mini-HTA, STA (Single Technology Assessment) and full HTA.**
    - The mini-HTAs are limited assessments performed by clinicians and supporting units within the hospitals.
    - The STAs focus on a single mode of health technology related to a comparator and are performed by either the Norwegian Medicines Agency (if the mode is a medicine) or the Norwegian Institute of Public Health (all other mode).
    - Full HTAs are broad assessments performed at the national level by the Norwegian Institute of Public Health. The full HTAs may for example be used to compare various methods that have been used in clinical practice for some time.
- When performing Full HTA assessments, the Norwegian Medicines Agency or the Norwegian Institute of Public Health works in close dialogue with clinicians that, among others, have been recruited by the four regional health authorities.

Norway – HTA pathway

An overview of the national system for managed introduction of new health technologies within the specialist service in Norway processes.

The map is not solely for ATMPs, but for all new health technologies in the specialist health care services.

The objective is to ensure that new technologies meet health needs and sustainability. Of the health care system, managed introduction and prioritization offer important tools.



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Norway – About NIPH

NIPH – Norwegian Institute of Public Health

- Norway's national public health institute is subordinate to the Ministry of Health and Care Services. NIPH acts as a national competence institution in public health in a broad sense for governmental authorities, the health service, the judiciary, prosecuting authorities, politicians, the media and the general public, international organizations and foreign governments.
- The Norwegian Institute of Public Health contributes to national and international Health Technology Assessments and performs the Full HTAs, pharmaceutical STAs and support mini-HTAs.

Mission

- To support decision makers in the health and welfare services by providing knowledge to help ensure high quality and equitable services.

Current activities of NIPH are divided into three main entities:

- HTA, systematic reviews and dissemination, teaching and support for EBM (Evidence-based Medicine) and evidence-based policy, clinical guidelines, The Grading of Recommendations Assessment, Development and Evaluation (GRADE) research and method development, method support to Cochrane, EPOC (Effective Practice and Organisation of Care Group) satellite, and secretariat for the Campbell Collaboration.
- Patient and user experience surveys, quality measurements and improvement in the health services, patient safety, and comparative health system analysis.
- Use of Norwegian electronic health library (Helsebiblioteket).

ATMP market entry from an HTA perspective

Danish perspective

Denmark - Overview

National HTA organization	Danish Medicines Agency (DKMA) / *Medicinrådet*
Purposes of HTA	Clinical practice guidelines and protocols, Planning and budgeting, Pricing of health products, Indicators of quality of care
How HTA is used in decision-making	Advisory, decision makers rely partly on the advice

HTA reviewed approved ATMP

Brand name	ATMP technology	HTA assessment of therapy	Payment method
Kymriah	GTMP	Not recommended as a standard treatment for relapses or refractory diffuse large-cell B-cell lymphoma after several systemic treatments. Recommended as standard treatment for B-cell acute lymphatic leukemia (ALL) who are refractory, in relapses following stem cell transplantation or in other or subsequent relapses.	No advanced payment method
Yescarta	GTMP	Not recommended as standard treatment for adult patients with relapses or refractory diffuse large-cell B-cell lymphoma after several systemic treatments.	No advanced payment method
Luxturna	GTMP	The Board of Medicines does not recommend the use of neparvovec as standard treatment for patients with hereditary RPE65-related retinal dystrophy.	No advanced payment method
Alofisel	sCTMP	Not recommended as possible standard treatment for Chron's Disease for some populations.	No advanced payment method
Holoclar	TEP	Recommended for some patient groups and not recommended for others.	No advanced payment method

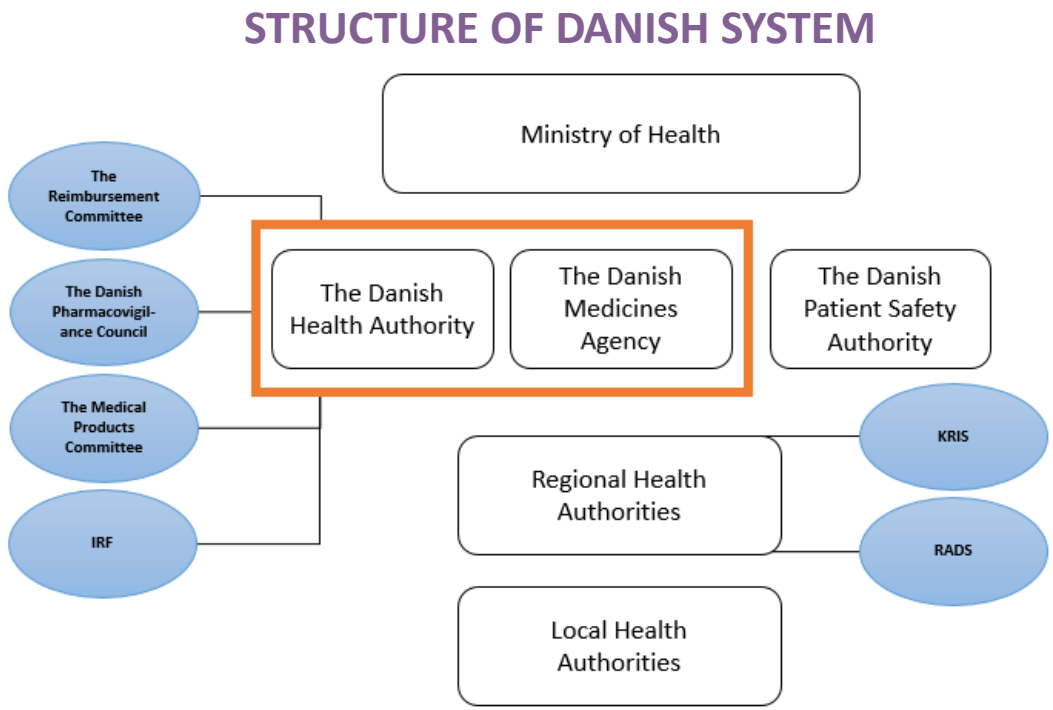
Denmark – Pricing and HTA methodology

Pricing

- Pharmaceutical companies do not have to apply for permission to set price for their medicines, there is no nationwide regulation for sales prices.
- Denmark, Norway and Iceland have started working together to negotiate prices on expensive pharmaceuticals.
- **We have not identified any new type of pricing model for approved the ATMPs.**

HTA methodology

- Denmark is in the process of reorganizing its HTA system through the new independent council Medicinrådet. Recent years it has been is decentralized after The Danish Centre for Health Technology Assessment department in the Danish Health and Medicines Agency closed in 2011.
- There is no regulatory mechanism in the Danish health service requiring the use of HTA in policy decisions, planning, or administrative procedures. Conclusions of HTA are often disregarded due to political or a health professional's priorities.
- A major concern about HTA is that the assessments are time consuming and thorough tasks. This can be difficult to fit into a short-term political process which often demands quick decisions



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ATMP market entry from an HTA perspective

Finnish perspective

Finland - Overview

National HTA organization	Finnish Coordinating Center for Health Technology Assessment (FinCCHTA)
Purposes of HTA	Clinical practice guidelines and protocols, planning and budgeting, reimbursement/package of benefits
How HTA is used in decision-making	Advisory, decision makers rely partly on the advice

HTA reviewed approved ATMP

Brand name	ATMP technology	HTA assessment of therapy	Payment method
Imlygic	GTMP	Recommended by quick mini-HTA	No advanced payment method - Fixed list prices
Kymriah	GTMP	Recommended (not by HTA)	No advanced payment method - Fixed list price of €330,000
Yescarta	GTMP	Recommended (not by HTA)	No advanced payment method -Fixed list price of €330,000
Alofisel	sCTMP	Recommended by quick mini-HTA	No advanced payment method -Not found

Finland – Pricing and HTA stakeholders

Pricing

- By 1st June 2019, 29 conditional reimbursement agreements have been made, 24 of which are in force. All the conditional reimbursement agreements made so far have been financial agreements in which cost-related uncertainty has been shared between a pharmaceutical company and society.
- Both the pharmaceuticals pricing board and the pharmaceutical companies have taken a favorable view of conditional reimbursements and it is likely that the parties wish the procedure to continue. However, a legislative change is required for its continuation.
- However, none of the conditional reimbursement agreements are used for ATMPs yet.

HTA is perormed by FinCCHTA

- FinCCHTA was established in 2018 to continue the HTA activities previously carried out by the National Institute for Health and Welfare's department FinOHTA. Finland closely follows EUnetHTA approaches.
- FinCCHTA participates in the international HTA collaboration, health services and HTA research including distribution and organization of the hospital-based HTA work within the national HTA-network (five university hospitals jointly producing the reviews). FinCCHTA collects all jointly produced reviews and gives national recommendations based on this work. FinCCHTA also produces systematic reviews and original research papers.
- FinCCHTA is a permanent member (one seat) in the Council for Choices in Health Care in Finland (COHERE), which issues recommendations on services that should be included in the range of public health services in Finland. The Council works in conjunction with the Ministry of Social Affairs and Health. COHERE can order HTA reports from FinCCHTA or redirect themes proposed to it to FinCCHTA.
- FinCCHTA is active in teaching and knowledge dissemination in HTA and research methodology.

ATMP market entry from an HTA perspective

German perspective

Germany - Overview

National HTA organization	Federal Joint Committee/ Gemeinsamer Bundesausschuss
Purposes of HTA	Pricing of health products, reimbursement/package of benefits
How HTA is used in decision-making	Mandatory, decision makers rely completely on the advice

HTA reviewed approved ATMP

Brand name	ATMP technology	HTA assessment of therapy	Payment method
Glybera	GTMP	Non-quantifiable added benefit	Withdrawn from market
Imlygic	GTMP	No added benefit	Handled as procedure - not AMNOG assessed (The Arzneimittelmarkt-Neuordnungsgesetz)
Kymriah	GTMP	Non-quantifiable added benefit	Pay-for-performance
Yescarta	GTMP	Non-quantifiable added benefit	Pay-for-performance, G-BA assessed
Luxturna	GTMP	On-going G-BA assessment	-
Provenge	sCTMP	Non-quantifiable added benefit	Withdrawn from market
Zalmoxis	sCTMP	Non-quantifiable added benefit	GKV reimburses with € 130 000/infusion
Alofisel	sCTMP	Non-quantifiable added benefit	Currently under price negotiation
Chrondocelect	sCTMP	Not eligible to early benefit assessment	Withdrawn from market
Holoclar	TEP	Not eligible to early benefit assessment	Not assessed; not reimbursed

Germany – Pricing

Pricing

- **Prices for ATMPs are negotiated at a national level**. Discount agreements are possible to reach since developers are free to conclude them with individual health insurers or associations of health insurance funds. These can include risk-sharing agreements and capitation agreements.
- An example of this is the health insurance service company GQW who signed a pay for performance risk-sharing agreement with Novartis for Kymriah, where Novartis will partially pay back the cost if the patient dies of his/her blood cancer within a defined period after the treatment.
- For the budget impact and affordability, Germany uses a two-step assessment where the HTA assessment is the first step and the second is a price negotiations with payers who have made an independent assessment. Rebates are common for new drugs as opposed to outcomes-based agreements, which are rare.
- **Germany has implemented pay-for-performance for some of the approved ATMPs, G-BA assessed (The Federal Joint Committee: 'G-BA' (Gemeinsamer Bundesausschuss).**

Germany- HTA stakeholders

- **The Ministry of Health: (Bundesministerium fuer Gesundheit) sets the framework for health care interventions**, approves measures taken within this framework, monitors the outcome of reforms and controls the work of the Statutory Sickness Funds. It is also the final decision-maker concerning reference pricing groups and reimbursement.
- **Federal Institute for Drugs and Medical Devices** (Bundesinstitut für Arzneimittel und Medizinische Produkte - BfArM) is an independent higher federal authority within the portfolio of the Federal Ministry of Health. One of the main tasks of the BfArM is the **authorization of finished medicinal products** on the basis of the German Medicines Act (Arzneimittelgesetz, AMG).
- The **German Institute for Health Technology Assessment: DAHTA** produces reports on medical, economic, social, ethical and legal issues related to the German health system, along with administering a database containing its own HTA reports, as well as national and international reports produced by other organizations. It provides information to interest groups and, together with scientific institutes, is involved in developing standards.
- **The Federal Joint Committee: 'G-BA' (Gemeinsamer Bundesausschuss**) is comprised of doctors, dentists, hospital representatives, representatives of the SHIs and patient representatives. The G-BA is the **central decision-making body concerning drug provision for those with statutory health insurance**. It regulates reimbursement and restrictions on prescribing on efficiency grounds. Furthermore G-BA assesses new methods of medical examination and treatment, evaluates and classifies new drugs on the German market and is responsible for the publication of treatment guidelines (submitted for approval to the Federal Ministry of Health).

Other HTA-related findings for Germany

- Orphan ATMPs benefit from special regulations where they are granted additional benefit, of relative lower burden of evidence compared to other medicines, by law if the annual sales do not exceed €50M.
- Extrapolation of data from new kinds of evidence than the traditional randomized controlled trials are frowned upon which makes it challenging to recognize and quantify long-term costs or potential outcome benefits.
- Germany have reimbursed six ATMPs, of which four is still on the market.

ATMP market entry from an HTA perspective

Dutch perspective

The Netherlands - Overview

National HTA organization	The National Health Care Institute/ Zorginstituut Nederland (ZINL)
Purposes of HTA	Reimbursement/ package of benefits
How HTA is used in decision-making	Results of HTAs have to be considered in decision-making process

HTA reviewed approved ATMP

Brand name	ATMP technology	HTA assessment of therapy	Payment method
Strimvelis	GTMP	Reimbursed as orphan drug	No advanced payment method
Yescarta	GTMP	Put in "waiting room" for expensive drugs	No advanced payment method
Holoclar	TEP	Reimbursed as orphan drug	No advanced payment method

The Netherlands - Pricing

Pricing

- A new pricing project is being tried,: the "no cure, no pay" model, in which certain expensive cancer drugs will be paid for only if they turn out to be effective after 16 weeks. However, **none of the ATMPs approved by EMA are under this scheme.**
- Netherlands are part of the joint initiative BeNeLuxA that works with drug pricing and reimbursement and is a collaboration between the governments of Belgium, The Netherlands, Luxembourg, Austria and Ireland. The initiative aims to ensure sustainable access to innovative medicine at affordable cost for patients.

HTA - methodology

- The National Health Care Institute (Zorginstituut Nederland, ZIN) conducts the HTA of pharmaceutical products in the Netherlands and makes recommendations for reimbursement in order to manage the basic health care package and to ensure that it contains all necessary care
- Whether pharmaceuticals are part of the basic package is assessed on the basis of our four package criteria: necessity, effectiveness, cost-effectiveness and feasibility.
- The HTA process usually starts with a request from the manufacturer. Before the official dossier is submitted often a draft dossier is discussed. From the submission of a complete final dossier to the reimbursement decision takes usually 90 days for outpatient drugs and 4 months for hospital drugs. Experts from ZINL write a report that is submitted for advice to the scientific advisory board (WAR).
- Thereafter, the Board of the ZINL sends a recommendation on reimbursement to the Minister of Health, Welfare and Sport (VWS) which makes the final reimbursement decision. Pharmaceutical products dispensed in community pharmacies are always assessed, hospital drugs are assessed if they have a major budget impact. The ZINL can also make a statement whether a care intervention, including pharmaceutical products, should be reimbursed by national insurance companies in a so-called position paper (guiding).

The Netherlands – Market approval stakeholders

- Before a drug can be introduced into the market it must be authorized by the **Medicines Evaluation Board** (College ter beoordeling van Geneesmiddelen) (MEB). The MEB is part of the Ministry of Health, Welfare and Sport.
- The MEB evaluates the drug based on criteria cited in the Medicines Act 2007 (Geneesmiddelenwet) and sets the conditions for authorizing the product for marketing in The Netherlands. The responsibility for the evaluation, authorization and pharmacovigilance of medicinal products for human use (including homeopathic and herbal medicines) rests with the MEB, which consists of doctors, pharmacists and scientists. The MEB has independent authority to take decisions on the availability of these medicinal products. The MEB is responsible for both the authorization and monitoring of effective and safe medicinal products and is jointly responsible for the approval of the medicinal products throughout the EU.

ATMP market entry from an HTA perspective

UK perspective

United Kingdom

National HTA organization	National Institute for Health and Care Excellence
Purposes of HTA	Clinical practice guidelines and protocols, Planning and budgeting, Indicators of quality of care
How HTA is used in decision-making	Advisory, decision makers rely partly on the advice

HTA reviewed approved ATMP

Brand name	ATMP technology	HTA assessment of therapy	Payment method
Imlygic	GTMP	Recommended with restriction	Patient access scheme
Strimvelis	GTMP	Gained full recommendation within its marketing authorisation via NICE's Highly Specialised Technology process	Patient access scheme
Kymriah	GTMP	Recommended by NICE	Reimbursed via Cancer Drugs Fund
Yescarta	GTMP	Recommended by NICE	Reimbursed via Cancer Drugs Fund
Luxturna	GTMP	Recommended by NICE	Patient access scheme, limited number of patients
Provenge	GTMP	Not recommended	Withdrawn from market
Alofisel	sCTMP	Not recommended	Not recommended
Chondrocelect	sCTMP	Recommended	Withdrawn from market
MACI	TEP	Recommended	Withdrawn from market
Holoclar	TEP	Recommended with restriction by NICE	Patient access scheme
Spherox	TEP	Recommended	Patient access scheme

United Kingdom - Pricing

Pricing

- 7 out of 10 ATMP drugs are reimbursed to date. Only one of these, Strimvelis®, was reimbursed at its full list price.
- Budgetary planning in the UK health service is not set up for potentially curative one-time therapies, such as gene therapies.
- In UK, Patient Access Schemes (PAS) are routinely used. Mostly, they are confidential discounts and in rare cases, they are outcome-based agreements.
- The UK uses fund-based payment models. An example of this is the Cancer Drugs Fund which can pay for new cancer drug even if they can be rejected by NICE.
- The UK information excludes Scotland as they have their own HTA system.

United Kingdom – HTA stakeholders and methodology

NICE – National Institute for Health and Care Excellence

- NICE was set up in 1999 to reduce variation in the availability and quality of NHS treatments and care. The institute began developing public health guidance in 2005.
- NICE's role is to improve outcomes for people using the NHS and other public health and social care services.
    - Producing evidence-based guidance and advice for health, public health and social care practitioners.
    - Developing quality standards and performance metrics for those providing and commissioning health, public health and social care services.
    - Providing a range of information services for commissioners, practitioners and managers across the spectrum of health and social care.
- The health economic evaluation in UK has a health system perspective where only direct costs to the health care system are considered as opposed to taking indirect costs of treatment and illnesses into account as well.

HTA Nice

- Health technologies can be appraised by NICE via two routes:
    - Multiple Technology Appraisal (MTA): MTAs examine a disease area or class of drugs and contain either new evidence gathered after the launch of a drug or include new economic modelling. The MTA process is based on input from a broad range of stakeholders, with emphasis on the Assessment Group who critically reviews the available evidence and produces an Assessment Report.
    - Single Technology Appraisal (STA): STAs have been developed to provide early guidance for new drugs targeting a single indication, as well as for new indications for drugs already on the market. This process has been developed to reduce the effect of NICE blight and drugs thought to improve life expectancy are likely to be prioritized. This process is more streamlined that the MTA process, with greater emphasis on the submission of evidence from the manufacturer. STA allows products which show plausible cost-effectiveness to enter into a Managed Access Agreement (MAA), whereby a restricted set of patients gain access to a treatment whilst more evidence is generated. This is pre- dominantly to the benefit of orphan oncology products.

ATMP market entry from an HTA perspective

French perspective

France - Overview

National HTA organization	Haute Autorité de Santé (HAS), Commission d'Evaluation des Médicaments, Agence Française de Securité Sanitaire des Produits de Santé (AFSSAPS), Transparency Commission (TC), Comité Economique des Produits de Santé (CEPS)
Purposes of HTA	Improve the quality of care delivered to patients
How HTA is used in decision-making	Usually clinical, in some cases health economic

HTA reviewed approved ATMP

Brand name	ATMP technology	HTA assessment of therapy	Payment method
Glybera	GTMP	Not recommended	Withdrawn from market
Kymriah	GTMP	Recommended	Price in negotiation / available through post ATU program* (Temporary Authorization for Use)
Yescarta	GTMP	Recommended	Reimbursed: Price set at € 327.000 for one injection with efficacy conditions in real life
Luxturna	GTMP	Recommended	Price in negotiation / available through post ATU program* (Temporary Authorization for Use)
Zalmoxis	sCTMP	Not recommended	Negative reimbursement decision
Alofisel	sCTMP	Recommended	Price in negotiation / available through post ATU program* (Temporary Authorization for Use)
Chondrocelect	sCTMP	Not recommended	Withdrawn from market
Holoclar	TEP	Recommended with restriction	Positive reimbursement decision / funding by hospitals

* Early access compassionate use program

France - Pricing

Pricing

- Agreements of price-volume and rebates are routinely negotiated for new medicines in France. Payment-by-result are rare, and it is limited to drugs in areas of clinical need when evidence at launch is not sufficient by usual HTA standards.
- If Comité Economique des Produits de Santé (CEPS) do not reach a price agreement with the developer, any of the parties can propose an established conditional price while further post-marketing data is collected.
- For the budget impact and affordability, France uses a two-step assessment where the HTA assessment is the first step and the second is price negotiations with payers who have made an independent assessment.
- Some of the ATMP have received what is called a temporary authorization for use (ATU), early access for compassionate use.
- **We have not identified any new type of pricing model for approved the ATMPs.**

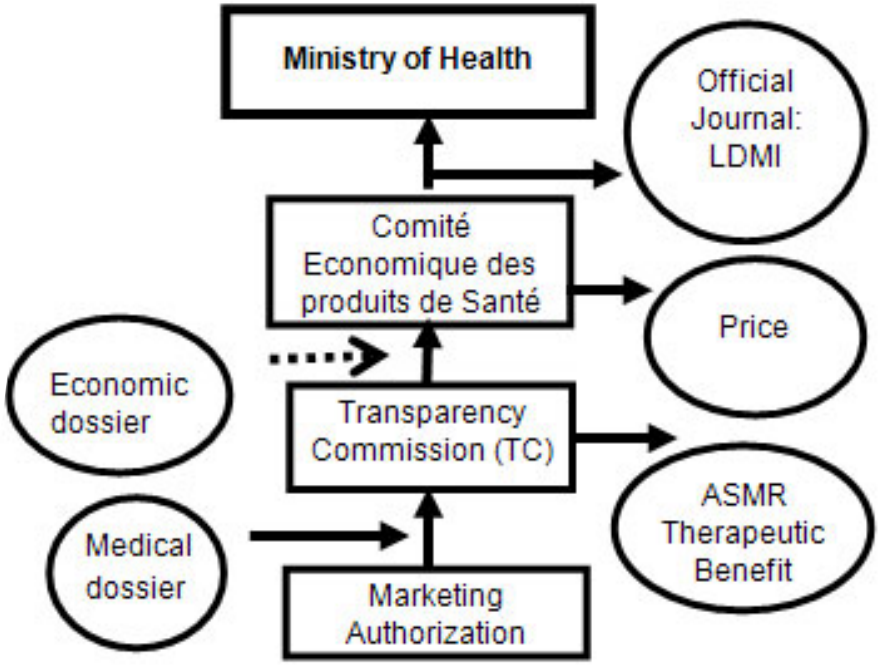
Other

- The French HTA bodies are highly driven by clinical evidence which makes them reluctant to provide access based on data available at the time of launch of an ATMP. Consequently, patients in need of therapy are not able to access new treatments in a timely manner. Two examples of this is with the cases of Glybera and Chondrocelect which were denied reimbursement in France due to uncertain clinical evidence.
- With the delayed reimbursement of a product as a background, collecting post-marketing evidence is very challenging for manufacturers.
- Four ATMPs have been recommended for reimbursement by French HTA bodies.

France – HTA methodology

- The general conditions of the reimbursement system are established by law and implemented principally at national level by governmental bodies.
- When marketing authorization is granted either by the EMEA or the French Medicine Agency (AFSSAPS), the company has to apply for reimbursement on positive lists to obtain funding by the mandatory health insurance (*assurance maladie obligatoire*).
- The decision-making process can be found to the right. Decision-making bodies are represented in boxes and the solid arrows are required steps in the process.

DECISION-MAKING PROCESS



France – HTA stakeholders

HAS – Haute Autorité de Santé

- The Haute Autorité de santé (HAS), the French national authority for health, is a consultative body providing independent scientific advice to the French public authorities. It was formed by the merger of ANAES (French National Agency for Accreditation and Evaluation in Health), the Transparency Committee, and the Committee for the assessment of devices and health technologies (CEPP) and FOPIM (Fund for the Promotion of Medical and Health Economics Information). The objective was to bring together into a single body all the expertise needed for patient-centered continuous quality improvement.

Mission

- HAS' mandate is to improve the quality of care delivered to patients through measures such as the production of good practice guidelines, the development of disease management programs for chronic conditions, continuing professional development (CPD), and accreditation of health care organizations.
- Practice appraisal becomes compulsory for all practitioners. HAS also assesses the expected and actual clinical benefit of drugs, medical devices, and diagnostic and therapeutic procedures and advises the authorities on their reimbursement. HAS will certify prescription software and compliance with the medical sales representatives' code of practice.
- HAS is governed by a board of eight members; the Board Chair is appointed by the Head of State. HAS may perform assessments on its own initiative or at the request of government (eg the Ministry of Health), national health insurance, learned societies, users' associations, etc. There are seven specialist committees, each chaired by a Board member. Each Board member is responsible for the policy, strategy and executive powers of their committee, and sets up working groups. Each Chair is supported by an operational manager who reports to the Director of HAS.

ATMP market entry from an HTA perspective

Spanish perspective

Spain - Overview

National HTA organization	Spanish Network of Agencies for Health Technology Assessment and Services of the National Health System
Purposes of HTA	Clinical practice guidelines and protocols, planning and budgeting, pricing of health products, indicators of quality of care, reimbursement/package of benefits
How HTA is used in decision-making	Advisory, decision makers rely partly on the advice

HTA reviewed approved ATMP

Brand name	ATMP technology	HTA assessment of therapy	Payment method
Imlygic	GTMP	Not found	Authorized, not commercialized yet
Kymriah	GTMP	Not found	Reimbursed; payment by results
Yescarta	GTMP	Not found	Reimbursed; payment by results
Chondrocelect	sCTMP	Recommended	Withdrawn from market
Alofisel	sCTMP	Not found	Reimbursed; payment by results
Holoclar	TEP	Not found	Commercialized, but not reimbursed

Spain - Pricing and reimbursement

Pricing and reimbursement

- Three therapies are reimbursed (Kymriah®, Yescarta® and Alofisel®; all of them with a payment by results agreement), one is commercialised but not reimbursed (Holoclar®); and the others are not commercialized. The three therapies that are reimbursed have been evaluated using Valtermed.
- When marketing authorization is granted either by the European Medicines Agency (EMEA) or the Spanish Medicine Agency AEMPS (Agencia Espanola del Medicamento y Productos Sanitarios), the Ministry of Health (MSC) initiates a procedure to decide on reimbursement of this new product on the national reimbursement list. The manufacturer is then invited to provide all relevant information to allow the Inter-Ministerial Pricing Commission CIPM (La Comisión Interministerial de Precios de los Medicamentos), led by MSC, to make a decision. If the outcome is positive (inclusion in the national reimbursement list), this decision is valid (mandatory) throughout the country.
- Many reimbursement decisions are made regionally which often causes the budget impact to outweigh other considerations in the evaluation of ATMPs.
- The view o pricing in Spain is that outcomes-based agreements, as well as expenditure cap agreements, can be reached between both the national and regional authorities with the pharmaceutical companies.
- Current legislation makes annuity payments impossible since committing for long-term spending for drugs is not allowed.
- **Spain has implemented new payment method, based on results.**

Spain - HTA stakeholders and methodology

- The National Health Service SNS (Sistema Nacional de la Salud) provides healthcare to the Spanish population. It is coordinated and supervised by the Ministry of Health and Consumer Affairs MSC (Ministerio de Sanidad y Consumo). Since the decentralization of healthcare to the seventeen autonomous regions (Comunidades Autónomas), the MSC focus more on pharmacovigilance, product approvals, cost-containment and long-term policies. The regions are responsible for the healthcare delivery and financing of it.
- **There are three different HTA levels in the country**:
  - national level for common benefit package excluding pharmaceuticals,
  - national level for pharmaceuticals, and
  - regional level.
- The HTA must include information about cost, efficacy, efficiency, effectiveness, safety and health care utility of the technology. The proposal of inclusion of new technologies that could significantly increase health expenditures also requires approval by the Fiscal and Financial Policy Council (Consejo de Politica Fiscal y Financiera Financial).
- The HTA for pharmaceuticals has a different process than that the HTA of other health care technologies.
- Due to the lack of either tools to cope with uncertainty in HTAs for ATMPs or experience and expertise, Spain as delayed ATMP assessments. This creates a problem with the time to access for patients in need of ATMPs.
- Access to gene therapy must follow the guidelines established in the "PLAN DE ABORDAJE DE LAS TERAPIAS AVANZADAS EN EL SISTEMA NACIONAL DE SALUD: MEDICAMENTOS CAR", included in it are besides other aspects:
- A network of reference centers for the use and administration of CAR-T therapies is being developed in Spain. Conditions are to be fulfilled by the centers for manufacturing CAR-T in Spain.

ATMP market entry from an HTA perspective

Italian perspective

Italy - Overview

National HTA organization	Italian Medicines Agency (AIFA) and national Agency for regional health services (AGENAS)
Purposes of HTA	Planning and budgeting, pricing of health products
How HTA is used in decision-making	Advisory, decision makers rely partly on the advice

HTA reviewed approved ATMP

Brand name	ATMP technology	HTA assessment of therapy	Payment method
Imlygic	GTMP	List Cnn: Not yet assessed	Not commercialized
Kymriah	GTMP	Information not found	Payment by results (ALL); obligatory discount (DLBCL)
Strimvelis	GTMP	List H: Hospital only	Reimbursed; payment by results
Zalmoxis	sCTMP	List H: Hospital only	Reimbursed; flat price per patient
Holoclar	TEP	List H: Hospital only	Reimbursed; payment by results

Italy - Pricing

Pricing

- Budget impact is one of the decision criterion for HTAs in Italy.
- Italy uses MEAs (Managed Entry Agreements), which have ensured faster ATMP assessments and market access than other countries in in the EU. These MEAs are used to control the spending on expensive and innovative products and take either of the three forms payment by results, cost-sharing or risk-sharing.
- **Kymriah, Strimvelis and Holoclar are currently available under a payment-by-result scheme** or what is also called Managed entry agreements. And Salmoxis has a flat cost per patient no matter of dosage.

Reimbursement

- In Italy, the general conditions of the reimbursement system are established on a national level and implemented at a regional level by governmental bodies.
- When marketing authorization is granted either by the European Medicines Agency (EMEA) or the Italian Medicine Agency AIFA (Agenzia Italiana del Farmaco), the company may apply for reimbursement on the National Pharmaceutical Formulary PFN (Prontuario Farmaceutico Nazionale). A product can be assigned to Class A, H or C.
    - Class A includes essential products and those intended for chronic diseases and are fully reimbursed by the national healthcare system.
    - Class H includes products that are only fully reimbursed in the hospital
    - Class C includes other products which do not have the characteristics of Class A and are not reimbursed.
- Drugs that get the classification *innovative*, may be paid by a separate fund and then be exempted from traditional budget caps and subsequently be included in regional formularies.
- That reimbursement decisions often are made regionally which often causes the budget impact to outweigh other considerations in the evaluation of ATMPs.

Italy – HTA stakeholders

The National Healthcare System SSN (Servizio Sanitario Nazionale) provides healthcare coverage to the Italian population. Although it is under the responsibility of the Ministry of Health, the system is decentralized resulting in three levels:

- National level: The Ministry of Health formulates every three years a healthcare plan PSN (Piano Sanitario Nazionale) that determines healthcare policies.
- Regional level: Twenty regions implement the PSN with their own resources and can adjust to region-specific needs. As a consequence, geographic disparity in terms of healthcare access or the level of co-payments exists.
- Local level: Local health units ASL (Azienda Sanitaria Locale) provide the health care services – e.g. primary medical services, coordination of all non-emergency admissions to public hospitals.

Reimbursement Organizations/HTA Organizations

- AIFA (Agenzia Italiana del Farmaco)
  Within the Italian Medicines Agency (AIFA), two committees are involved in the pricing and reimbursement procedure for pharmaceuticals.
- CTS (Comitato Scientifico e Tecnico)
  This committee makes a decision on the reimbursement, local marketing authorization and positive list revisions.
- CPR (Comitato Prezzi e Rimborso) This body will assess the manufacturers applications, collect information from the National Observatory on the Use of Pharmaceuticals (OsMED) and will negotiate reimbursement with the manufacturers.

When AIFA considers a new drug, level of innovation, unmet need, added therapeutic benefit and evidence quality is considered. Orphan drugs are partly or fully exempted from the evidence quality criterion and new rules from 2017 are designed to faster and more streamlined access throughout the country.

ATMP market entry from an HTA perspective

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Okee is a seasoned life science and healthcare professional with many years of experience and expertise gained from working on all sides of the industry.

Okee is a creative and innovative professional with 15 years of experience from positions including strategy and management consultant, venture capital investor, analyst, IR communicator to name a few, foremost within the life science and healthcare sector.

Prior to MSC, Okee worked as an independent management consultant and senior advisor primarily with Nordic biotech and healthcare companies where he assisted them in understanding changes, finding partners and creating value. Additionally, Okee has a background from the VC industry as a portfolio manager for Allanex, a Swedish VC firm focused on medical device and biotech companies. Previous positions includes venture manager at BillerudKorsnäs Venture, IR manager at Elekta, equity analyst at Carnegie and analyst at Boston Consulting Group.

Okee has a scientific background and an MBA from the Stockholm School of Economics, and combines a unique understanding of research with profound experience from the business side of running a corporation, an aspect which is often overlooked or undervalued.

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