Presenters

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Cell & Gene Therapy products are approved for use in the US

Product	Manufacturer	Indication	Approval
Provenge® (sipuleucel-T)	Dendreon	Prostate cancer	Apr. 29, 2010
Yescarta® (axicabtagene ciloleucel)	Gilead	DLBCL	Oct. 18, 2017
Imlygic® (talimogene laherparepvec)	Amgen	Melanoma	Oct. 27, 2015
Kymriah® (tisagenlecleucal)	Novartis	ALL and DLBCL	Aug. 30, 2017
Luxturna™ (voretigene neparvovec-rzyl)	Spark	Retinal dystrophy	Dec. 19, 2017
Zolgensma® (onasemnogene abeparbobec-xioi)	Novartis	SMA	May 24, 2019

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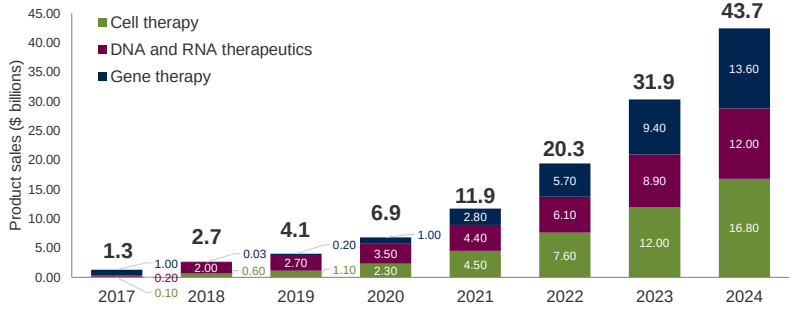
Cell & Gene Therapy expected to be a $43.7B market in 2024

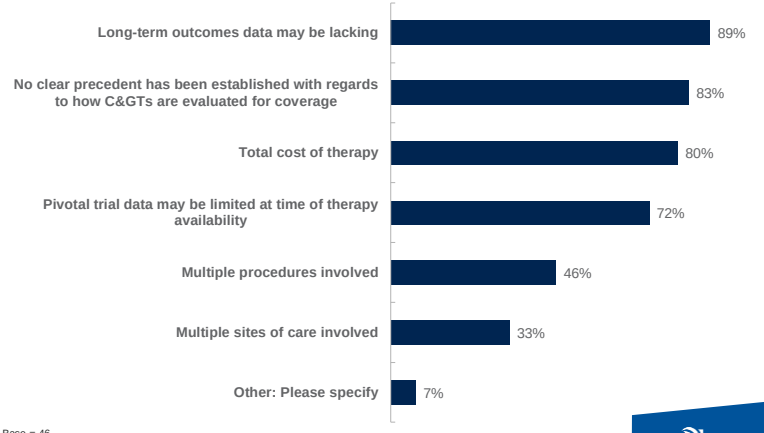
Sales Growth Trends of Cell, Gene, and Nucleic Acid Therapy Products from 2017 to 2024



Adapted from EvaluatePharma, March 2019

Xcenda Payer Research

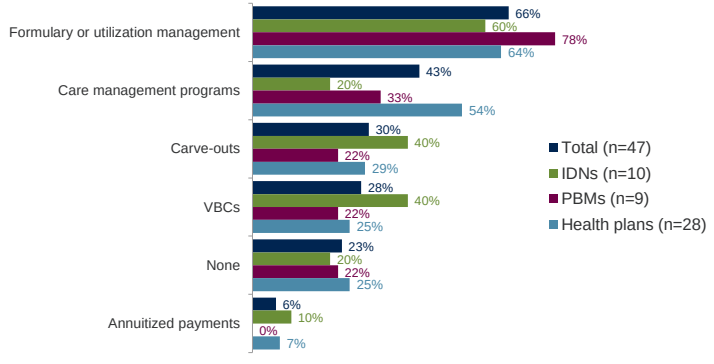
Significant Impediments to Cell & Gene Therapy Coverage



Base = 46
q28: Which of the following characteristics impede/would impede coverage significantly with regard to C&GTs?

Xcenda Payer Research

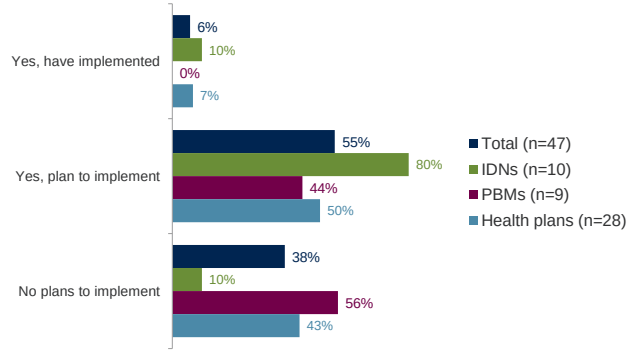
Types of Experiences with Cell & Gene Therapy



Q36. Which of the following types of experiences does your organization have designing benefits or payment models for cell and gene therapies (eg, high investment)?
IDNs: integrated delivery networks; PBMs: pharmacy benefit managers; VBC: value-based contracting

Xcenda Payer Research

Plans for Value-Based Contracts for High-Investment Medications



Q37. Has your organization implemented or do you have plans to implement any specific value-based contracts for high-investment medications (eg, cell or gene therapy, CAR-Ts)?
IDNs: integrated delivery networks; PBMs: pharmacy benefit managers

New payment models are being tested to address the cost of C&GT

Pay-for-Performance	Pay-Over-Time	Re-Insurance/Risk Pools
Benchmarking future payments on positive health outcomes for patients or providing rebates in cases in which the therapy was not as efficacious as expected	Payers amortize the cost of therapies over several years to better reflect the value provided by cell and gene therapies	Risk is shared by multiple insurance companies

Key Considerations

- Understand no one size fits all strategy
- Engage payers early during pre-approval
- Develop a market access plan with aligned evidence tactics
- Plan ahead for multimarket support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Can We Afford Curative Therapies Without Sharing Risks?
Insights from the UK
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ACCELERATING PATIENT ACCESS
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ATMP regulatory framework

Committee for Advanced Therapies (CAT) – ATMP Classification

Committee for Advanced Therapies (CAT) – Draft Marketing Authorisation Opinion

Committee for Human Medicinal Products (CHMP) – Review Draft Opinion

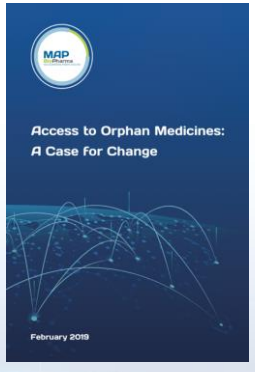
EMA – Final Marketing Authorisation

Approx. 210 days (excluding clock-stop)

Pricing precedents show HTAs of ATMPs are held to the same cost-effectiveness measures as for conventional therapies

Examples:

- Strimvelis (NICE HST 7)
  - High-cost: €594,000
  - Little data available, but potential QALY gain is huge – one-off treatment with lifetime benefit
  - Therefore, highest plausible ICER was £120,506 (within HST threshold)
- Tisagenlecleucel (Kymriah) (NICE TA 567, SMC 2129)
  - £282,000 per infusion
  - Patient access scheme (PAS) needed in Scotland to qualify as cost-effective
  - Managed Access Agreement (MAA) and Cancer Drugs Fund (CDF) needed in England (reappraisal needed in future based on additional data)
  - Did not qualify for HST so held to £30,000/QALY threshold



MAP Online news on ATMPs in the NHS: https://mapbiopharma.com/home/2018/12/nice-gives-final-seal-of-approval-for-gileads-car-t-in-non-hodgkin-lymphoma/
HST: Highly specialised technologies

ATMPs in England between 2017 and 2019 – guidance is positive, but often restricted (NICE sometimes refer to this as 'optimised')

ATMP	Type	Indication	NICE HTA	Notes
Luxturna (voretigene neparvovec) [Spark]	Vector replacing defective *RPE65* alleles with a functional copy (Gene therapy)	Inherited retinal dystrophy	HST: Guidance (recommended with simple discount patient access scheme) 09 Oct 2019	Orphan designation
Kymriah (tisagenlecleucel) [Novartis]	CAR-T (Somatic cell therapy)	Acute lymphoblastic leukaemia (ALL) B-cell lymphoma (DLBCL)	STA (ALL): Recommended (via CDF) 21 Dec 2018 STA (DLBCL): Positive FAD (via CDF) 01 Feb 2019 (following negative ACD, 19 Sept 2018)	PRIME (23 June 2016); Orphan designation
Yescarta (axicabtagene ciloleucel) [Gilead (Kite)]	CAR-T (Somatic cell therapy)	B-cell lymphoma (DLBCL & PMBCL)	STA (DLBCL): Recommended (via CDF) 23 Jan 2019 (following negative ACD, 28 Aug 2018)	PRIME (26 May 2016); Orphan designation
Strimvelis [GSK]	Bone marrow-derived CD34+ cells edited to contain functional *ADA* genes (Gene therapy)	ADA-SCID	HST: Guidance (recommended) 07 Feb 2018	Orphan designation
Spherox (chondrosphere) [CO.DON]	Transplant of cultured cells (Tissue engineering)	Articular cartilage defects	STA: Guidance (restricted) 7 Mar 2018	
ChondroCelect (autologous chondrocyte implantation) [TiGenix]	Transplant of cultured cells (Tissue engineering)	Articular cartilage defects	STA: Guidance (restricted) 04 Oct 2017	
Holoclar [Chiesi]	Cultured corneal stem cells (Tissue engineering)	Corneal stem cell deficiency	STA: Guidance (restricted) 16 Aug 2017	Orphan designation

Some surprises/inconsistency in routing to single technology appraisal vs highly specialised technology appraisal – **Proposed as HST**

AveXis

- Onasemnogene abeparvovec for treating spinal muscular atrophy type 1 [ID1473]
- Expected publication date: 08 September 2020

Orchard Therapeutics

- OTL-101 for treating adenosine deaminase deficiency–severe combined immunodeficiency [ID1152]
- Expected publication date: TBC

Some surprises/inconsistency in routing to single technology appraisal vs highly specialised technology appraisal – **Proposed as STA**

Atara Bio
- ATA129 for treating post-transplant lymphoproliferative disorder caused by the Epstein-Barr virus ID1203
- Expected publication date: TBC

bluebird bio
- Zynteglo for treating transfusion-dependent beta-thalassaemia ID968
- Expected publication date: 24 June 2020

Gliovac, Epitopoietic Research Corporation
- ERC1671 for treating progressed or recurrent glioblastoma ID1623
- Expected publication date: TBC

Kiadis Pharma
- ATIR101 with haploidentical haematopoietic stem cell transplantation for haematological cancers [ID1093]
- Expected publication date: TBC

Liso-cell, Celgene
- Lisocabtagene maraleucel for treating large B-cell lymphoma after at least 2 therapies ID1444
- Expected publication date: 09 December 2020

Northwest Biotherapeutics
- DCVax-L for treating newly diagnosed glioblastoma multiforme [ID836]
- Expected publication date: TBC

Implications: other than regulatory requirements, ATMPs are not considered different to conventional therapies in UK

- **Must** demonstrate cost-effectiveness
- No special allowances, ATMPs need to fit to existing framework
- It's becoming understood that **high one-off cost** can be offset by **long-term/lifelong effects**, but decision makers need the evidence to show this
- Use of PAS, commercial access agreements, managed access arrangements, etc, may be needed to meet thresholds (as with other high-cost drugs)

MAP Online review of the first eight ATMPs in the UK Presented at ISPOR 20th Annual European Congress; Glasgow, Scotland; 4-8 November 2017. Presentation Code: PHP57

Outlook for ATMPs in the UK: Opportunities ahead?

- NICE committees already highlight ‘innovation’
- NICE methodologies review:
  - New modifiers (similar to ‘end-of-life’)
  - Improved ways to handle uncertainty in treatment effect
- New Commercial Framework (at NICE and NHS England separately) may open different types of agreements enabling patient access
- Good appreciation by UK decision makers of issues facing ATMPs. They openly discuss:
  - Novel trial designs
  - Fewer RCTs (more single-arm studies etc)
  - Conditional approval/approval under exceptional circumstances

Presented at ISPOR Europe 2019; Copenhagen, Denmark; 2-6 November, 2019; Poster PNS215

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Can We Afford Curative Therapies Without Sharing Risks?

Insights from France

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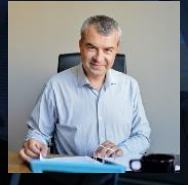

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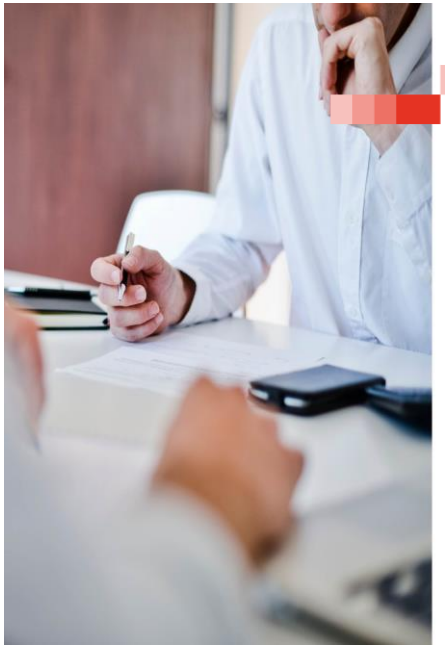

ATMPs assessment and funding in France

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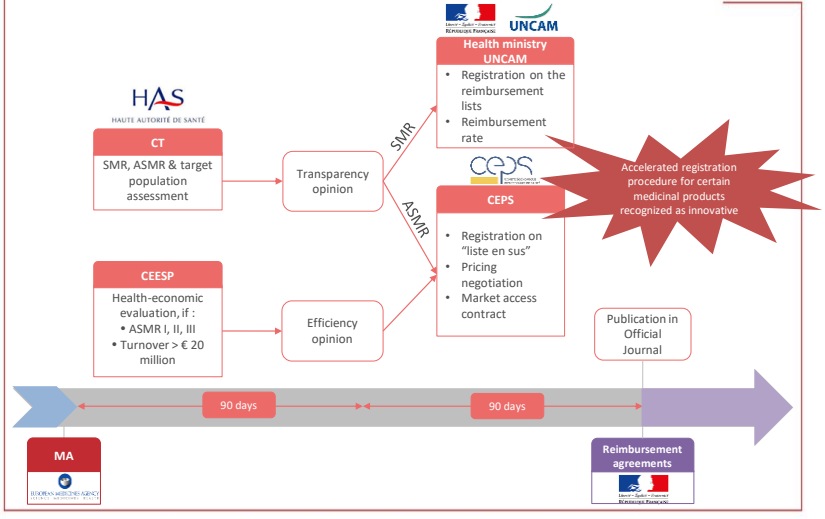
Context

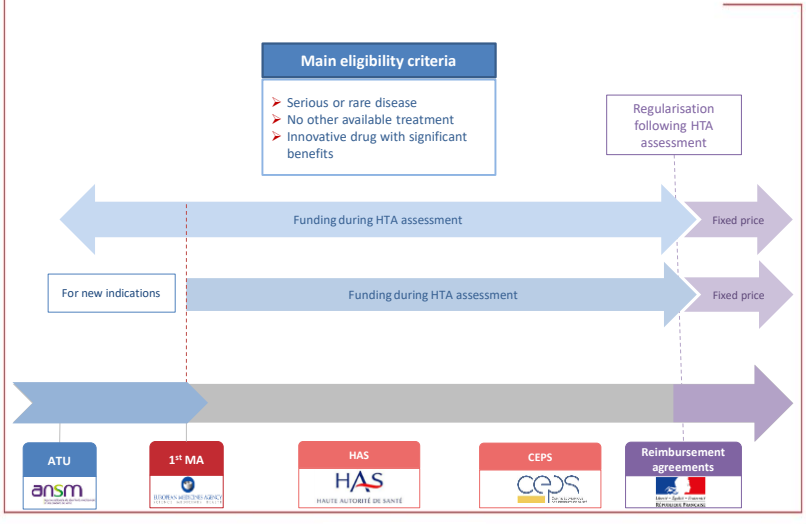
- Advanced therapy medicinal products (ATMPs), comprising gene therapies, tissue engineered products and somatic cell therapies, have the potential to reshape the treatment of a wide range of conditions, particularly in disease areas where conventional approaches are inadequate
  - ➔ No clinically relevant comparators
  - ➔ Low target population
  - ➔ Mostly orphan medicine status
- Hospital administration
  - ➔ For most drugs ➔ funding within the DRG
  - ➔ For innovative ones ➔ registration on « Liste en sus » ➔ funding in addition to DRG (pricing negotiation with CEPS)
- Access to innovative treatments raises the question of adapting the regulatory and pricing mechanisms for ATMP in France
  - ➔ Significant risk of restricting access to innovative treatments because of their high price
  - ➔ Discordance between ATMP high price and public budget constraint
  - ➔ Regulation mechanism with fixed price intervening even though the drug has not been evaluated under real-life conditions

HTA assessment ➔ same criteria and process

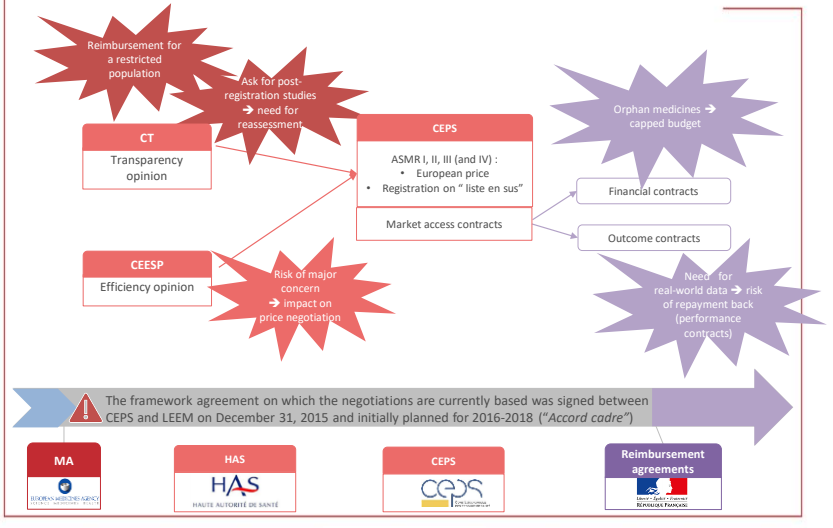


SMR: clinical benefit, in 4 levels from insufficient (no reimbursement) to important
ASMR: improvment of clinical benefit, in 5 levels from none (V) to major (I)

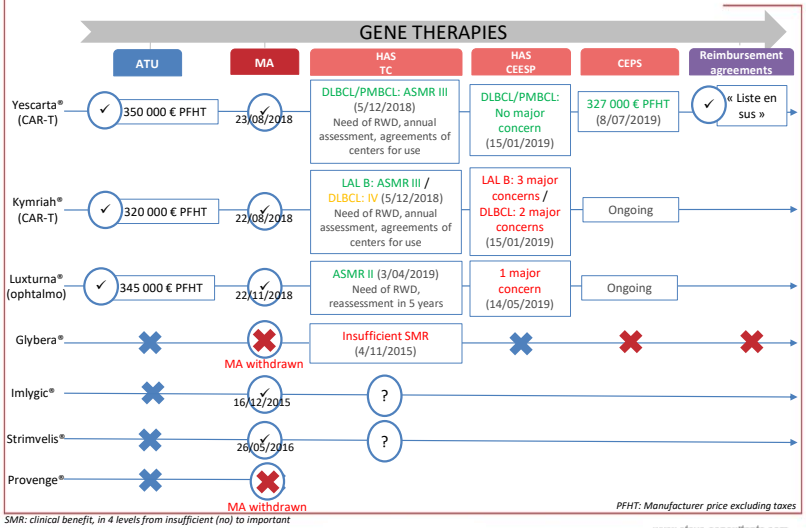
ATMPs: Potential early market access and funding



Additional request, pricing and market access contracts



The beginning of ATMP assessment in France (1/2)



SMR: clinical benefit, in 4 levels from insufficient (no) to important
ASMR: improvment of clinical benefit, in 5 levels from none (reimbursementV) to major (I)
PFHT: Manufacturer price excluding taxes

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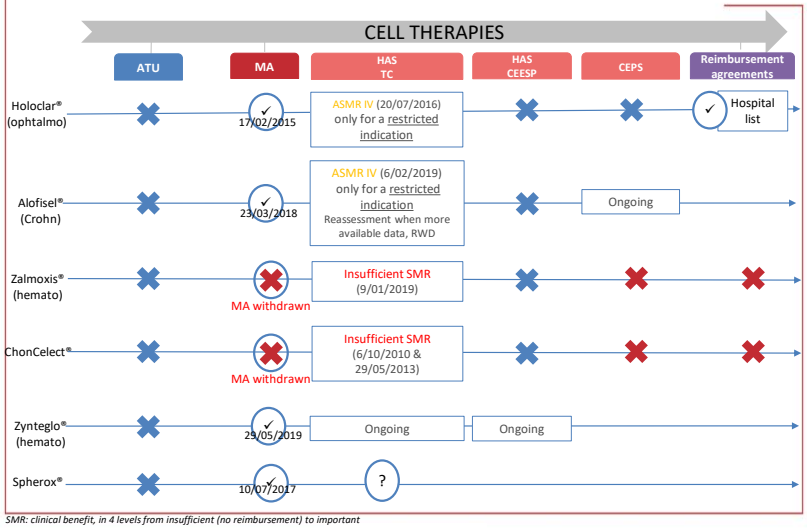
The beginning of ATMP assessment in France (2/2)



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Conclusion

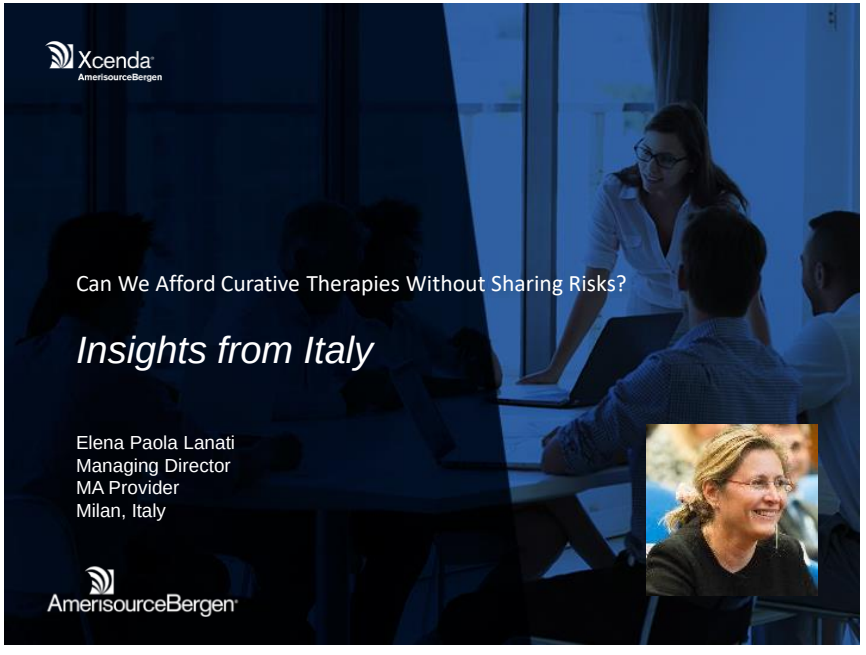
Statements

- For all drugs: same process and criteria for assessment
- Opportunity for early access and funding for ATMP if medical need is unmet
- Eligibility criteria for registration on « liste en sus » not always relevant for ATMP (clinical development, comparators…)
- A new framework agreement (« *Accord cadre* ») should soon be signed between CEPS and LEEM (*conditional reimbursement?*)

➔ New economic and organizational models to be developed for innovative products

Recommendations

- Favor early and in-process dialog with authorities
- Evaluate for Temporary Authorization for Use (ATU)
- Anticipate RWD and authorities' requests for post-registration studies (unmet need, target population, post-registration study and follow-up)
- Anticipate health-economic evaluation and its impact on price negotiation and market access contracts with CEPS



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Pricing, Reimbursement, and Access of ATMPs in Italy

Elena Paola Lanati
5 November 2019

Italy has been a pioneering country with ATMPs, but reimbursement is slowing down

ATMPs	Innovativeness	Reimbursement	List price*	Agreements
Holoclar	x	✔	€ 95.000	- payment by result - registry
Imlygic	Request not submitted			
Strimvelis	✔	✔	€ 594.000	- payment by result - registry
Zalmoxis**	x	✔	€ 149.000	- Flat price/patient - Registry
Spherox	Request not submitted			
Alofisel	-	Class C	-	-
Kymriah	in both indications	✔	€ 320.000	- DLBCL: hidden discount, payment at result (6 and 12 months), registry - ALL: **payment at result** (6 and 12 months), registry
Yescarta	Under CPR assessment			
Luxturna	Under CTS assessment			
Zynteglo	Under CTS assessment			

*VAT excluded; **announced withdrawal

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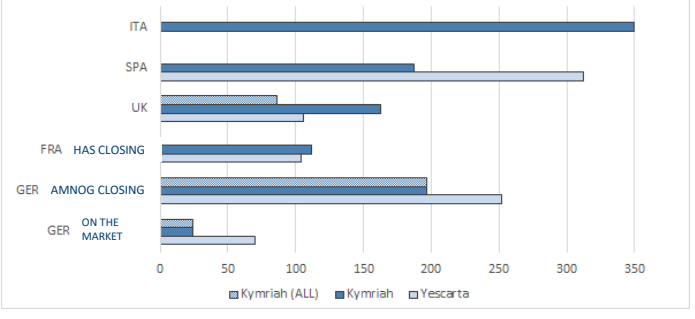
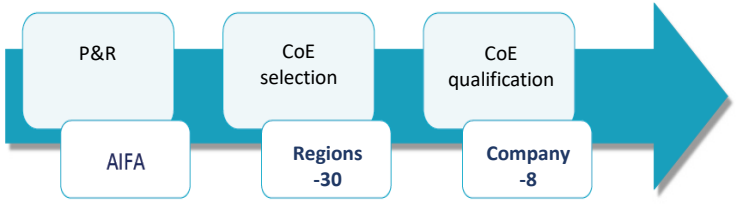
CAR-T P&R assessment in Italy was the longest and has not concluded yet



Access scheme for CAR-T in Italy

For the first time, AIFA defined minimum criteria for Kymriah Center of Excellence choice



CRITERIA FOR REGIONAL CHOICE FOR CAR-T CENTER

1. National Transplant Center certification, according to EU law
2. JACIE accreditation for allo-transplant including clinical unit, collecting and processing units
3. Intensive care and medical emergency units
4. Proper multidisciplinary team for clinical and complications management

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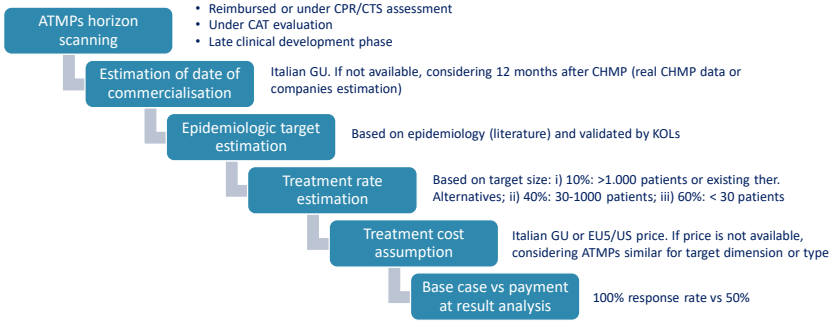
Model developed to assess budgetary impact of ATMPs

Based on literature, data, and assumptions



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17 ATMPs were included for 16 diseases

	Disease	ATMPs	Status	CHMP (*estim.)	GU (*estim.)	Target population	Treatment rate	Target /year	Treatment cost (list price)	Cost rationale
1	ADA-SCID	Strimvelis OTL-101	H Fase II	Apr 2016 S1 2021*	Aug 2016 S1 2022*	2	60%	1	596.000 €	GU Strimvelis
2	GvHD in case of HSCT for hema. malignancies	ATIR-101	Under CAT	S1 2020*	S1 2021*	508	10%	51	149.000 €	GU Zalmoxis
3	Perianal fistulas in CD	Alofisel	C	Dec 2017	S2 2020*	1.012	10%	101	60.000 €	Spain Price
4	ALL	Kymriah	H	June 2018	Aug 2019	40	40%	16	320.000 €	GU Kymriah
5	DLBCL + PMBCL	Kymriah Yescarta Liso-cel	H CPR assessment Phase II/III	June 2018 June 2018 S1 2021	Aug 2019 S1 2020 S1 2022	700	40%	280	320.000 €	GU Kymriah
6	Retinal distrophies	Luxturna	Valutazione CTS	Sept 2018	S1 2020*	70	40%	28	345.000 €	Germany price/eye
7	β-thalassemia	Zynteglo	Valutazione CTS	Mar 2019	S2 2020*	1.200	10%	120	1,575 M €/ 5 years: 315.000 €/year	US price & agreements
8	SMA 1	Zolgensma	Valutazione CAT	S1 2020*	S1 2021*	27	60%	16	1,936 M €/ 5 years: 387.290 €/year	US price & agreements
9	Haemophilia A	Valrox	Phase II/III	S2 2020*	S2 2021*	1.850	10%	185	320.000 €	GU Kymriah
10	Haemophilia B	SPK-9001	Phase III	S1 2021*	S1 2022*	898	10%	90	320.000 €	GU Kymriah
11	MM	Ide-cel	Phase II/III	S1 2021*	S1 2022*	1.800	10%	180	320.000 €	GU Kymriah
12	CALD	Lenti-D	Phase II/III	S1 2021*	S1 2022*	3	60%	2	1,575 M €/ 5 years: 315.000 €/year	Zynteglo US price (same Company)
13	MLD	OTL-200	Phase II	S1 2021*	S1 2022*	2	60%	1	596.000 €	GU Strimvelis
14	AADC deficiency	AAV-hAADC-2	Phase II	S1 2021*	S1 2022*	60	40%	24	320.000 €	GU Kymriah
15	Duchenne Dirtrophy	SRP-9001	Phase II	S1 2022*	S1 2023*	68	40%	27	320.000 €	GU Kymriah
16	Stargardt Disease	SAR422459	Phase II	S2 2023*	S2 2024*	1.293	10%	129	320.000 €	GU Kymriah

GU: official gazette ex-factory price

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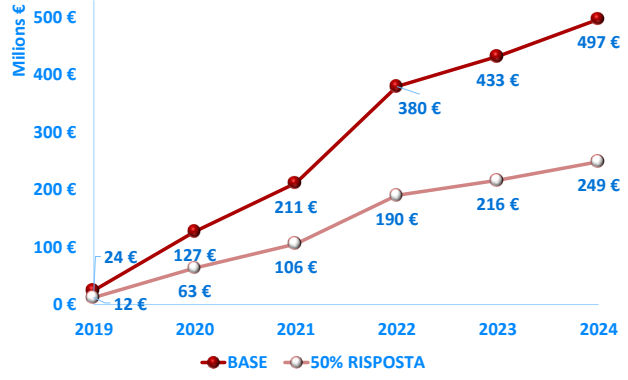
ATMPs expected BI grows from 24 M € in 2019 to 497 M € in 2024 (base case)

In payment at result scenario we assume 50% success rate



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Proposals to deal with ATMP in Italy

KEY PRIORITIES:

1. **Public-private working group** involving all stakeholders to plan common actions according to ATMPs horizon scanning, and related opportunities, issues and organizational impact
2. **Early dialogue** with decision makers to accelerate ATMPs access
3. Find **innovative and flexible reimbursement scheme** according to the specific ATMP ad disease
4. **Policy makers involvement** in strategic planning to attract ATMPs manufacturing and R&D in Italy
5. Invest in specific **training at medical-scientific universities to develop ATMP competencies and also technology transfer**

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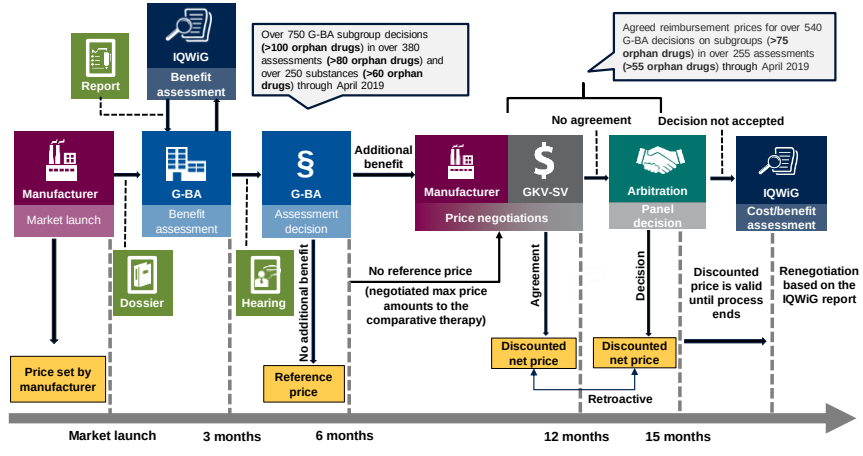
AMNOG (Arzneimittelmarkt-Neuordnungsgesetz) Implemented in 2011



Xcenda database: No guarantee of completeness, including discontinuations, etc.
Key: G-BA – Federal Joint Committee (Gemeinsamer Bundesausschuss); GKV-SV – Head Organisation of SHI (Spitzenverband Bund der Krankenkassen); IQWiG – Institute for Quality and Economics in Healthcare System (Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen).

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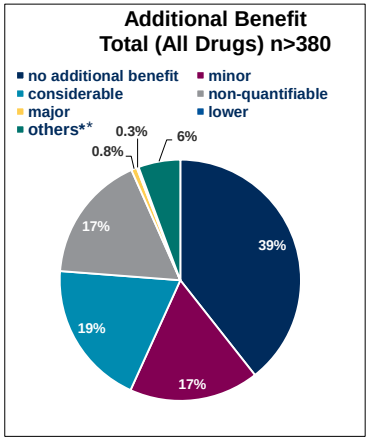
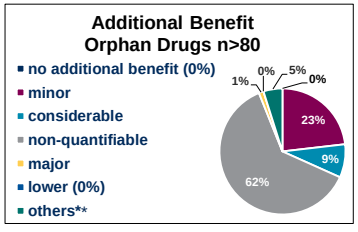
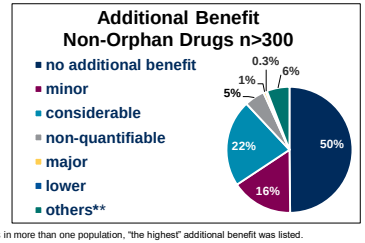
AMNOG – Granted Additional Benefits and Orphan Drugs

Assessment* level (April 2019)







Xcenda database: No guarantee of completeness, including discontinuations, etc.
* All separate assessments were included. If the G-BA decision statement indicated additional benefits in more than one population, "the highest" additional benefit was listed.
Additional benefit hierarchy: lower < no additional benefit < non-quantifiable < minor < considerable < major.
** Others = exemptions / stopped proceedings (including 50 Mio orphan limit) / not covered by §35a / reference price group / not listed anymore.

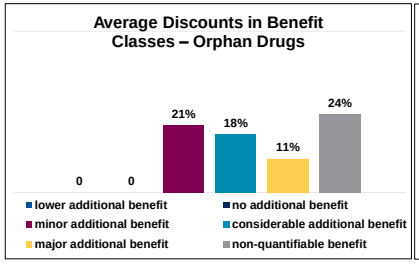
AMNOG – Price Negotiations and Orphan Drugs

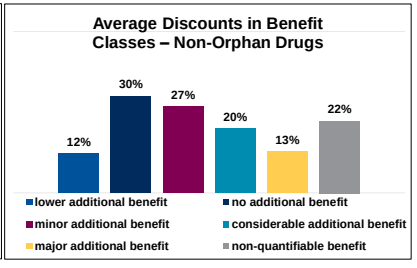
Assessment* level (April 2019)

Published discounts were taken from the Lauer tax**

€

- For n>255 (>200 non-orphan drugs and >55 orphan drugs) subgroups, discounts were listed
- 0% assessed orphan subgroups with no additional benefit (46% non-orphan drugs)
- 27% with minor additional benefit (18% non-orphan drugs)
- 8% with considerable additional benefit (30% non-orphan drugs)
- 2% with major additional benefit (1% non-orphan drugs)
- 63% with non-quantifiable benefit (5% non-orphan drugs)
- 0% with less additional benefit (0% non-orphan drugs)





Xcenda database: No guarantee of completeness.
* All separate assessments were included. If the G-BA decision statement indicated additional benefits in more than one population, "the highest" additional benefit was listed.
Additional benefit hierarchy: lower < no benefit < non-quantifiable < minor < considerable < major.
** Lauer tax: https://www.cgm.com/lauer-fischer/index.de.jsp

Advanced Therapeutic Medicinal Product (ATMP) Submissions Under the AMNOG Regulation

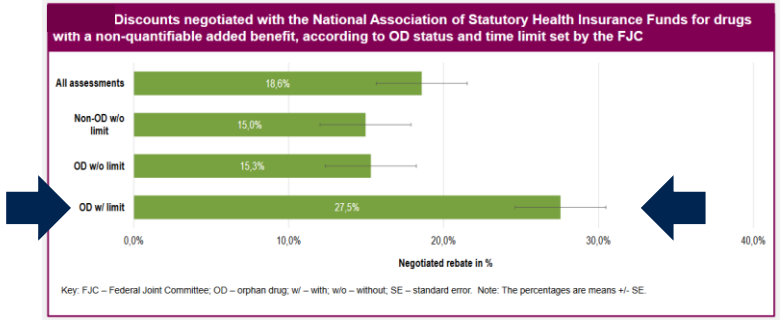
Small populations? Yes, but they're not super small!

Brand name	Indication	Orphan status	Number of patients according to G-BA assessment	Start of AMNOG procedure / date of market entry	Result of AMNOG assessment	Term set by G-BA (duration)
Gene therapeutics						
Glybera®	Familial lipoprotein lipase deficiency	Yes	-	01.11.2014	Not quantifiable	Yes (2.5 years)
Imlygic®	Unresectable melanoma	No	375–670	15.06.2016	No additional benefit	No
Kymriah®	ALL, DLBCL (lymphoma)	Yes	440–700	15.09.2018	Not quantifiable	Yes (1 year)
Luxturna®	Innate vision loss	Yes	100–530	15.04.2019	Considerable benefit	Yes (2.5 years)
Provenge®	Metastatic, castrate resistant prostate cancer	No	Unknown	01.10.2014	Not quantifiable	Yes (3 years)
Yescarta®	DLBCL (lymphoma)	Yes	475–709	01.11.2018	Not quantifiable	Yes (3 years)
Somatic cell therapeutics						
Alofisel®	Stem cell transplantation	Yes	90–230	01.06.2018	Not quantifiable	No
Zalmoxis®	Stem cell transplantation	Yes	100–140	15.01.2018	Not quantifiable	Yes (3 years)

Key: ALL – acute lymphatic lukemia; AMNOG – Pharmaceuticals Market Reorganisation Act; G-BA – Federal Joint Committee; DLBCL – diffuse large B-cell lymphoma.

Is the Federal Joint Committee (G-BA) Putting More Pressure on Manufacturers?

Are Price Discounts Impacted by the Uncertainty of Available Data at Market Launch?!



Xcenda Database: Presented at: ISPOR 20th Annual European Congress | 4-8 November 2017 | Glasgow, Scotland | Session IV | PHP148.

Value-Based Pricing Schemes Emerged Instantly

Hard clinical outcomes are needed to measure maintenance/success

Novartis Pharma GmbH und GWQ ServicePlus schließen Vertrag über ein innovatives Erstattungsmodell für die CAR-T-Zelltherapie

Nürnberg, 06. März 2019 – Die Novartis Pharma GmbH und die GWQ ServicePlus AG (Gesellschaft für Wirtschaftlichkeit und Qualität bei Krankenkassen) realisieren für die Erstattung von Gentherapien ein Pilotprojekt und haben für die Kymriah®-CAR-T-Zelltherapie einen Vertrag über ein innovatives Erstattungsmodell (Pay for Outcome) abgeschlossen.

FiercePharma

Bluebird prices gene therapy Zynteglo at €1.575M in Europe, to be paid over 5 years

bluebirdbio

PHARMAZEUTISCHE ZEITUNG

Neue Therapien

Erster Rabattvertrag zu Yescarta

Unter Federführung der DAK haben sich die Ersatzkassen mit dem US-Pharmaunternehmen Gilead auf einen Rabattvertrag zu dem CAR-T-Zelltherapeutikum Yescarta® geeinigt. Der Vertrag richte sich nach dem Outcome für die Patienten in puncto Überleben, wie ein DAK-Sprecher auf Anfrage der PZ bestätigte. Nähere Vertragsdetails sind bisher nicht bekannt.

Outlook for Pricing of ATMPs in Germany

Changes will surface on 2 levels: assessment of evidence by regulators and affordability to payers

Regulators

- G-BA will start to implement mandatory disease registries in cases of non-conclusive evidence at time of market launch
  - G-BA will be part of setting up the methods and requirements for individual registries
  - This will become standard for ATMPs
  - ATMP price will be significantly reduced over time, if a company is non-compliant in setting up a real-world registry

Payers

- Use of real-world evidence data will rise significantly
- Individual health insurance organizations will use additional pay-for-performance contracts more widely
- Pay-for-performance agreements linked to clinical outcomes of a drug will rise (discounts are confidential)

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Pricing and Affordability for Targeted Therapies

Targeted therapies will fulfill high unmet clinical needs!

Regulators

- Regulatory approval is clearly directed towards targeted therapies, and thus, trying to **maximize patient-individual benefits regardless of affordability issues**
- **Local country regulators are still struggling** with “non-traditional” and in their eyes “immature” data packages at initial launch

Providers

- Clinicians are **eagerly waiting for targeted therapies**, especially in areas with high unmet need
- Slower adoption compared to classic innovations must be expected due to **uncertainty of long-term effects, safety, and cost**
- Providers may also **carry financial risk in many countries** for prescribing these type of therapies, requiring market-specific targeted communication

Pricing and Affordability for Targeted Therapies

The pharma industry must be the driver in addressing affordability challenges!

Payers

- As therapies potentially offering a cure may be cost-effective under traditional terms, **affordability** must be **discussed openly**
- Payers expect **the industry to provide solutions** on a country-by-country basis
- Conditional reimbursement likely to be linked to in-market **real-world evidence** data collection on a country-by-country basis

Pharma

- Industry must be **proactive in providing country-fitting tools** to address affordability
- Every product requires a unique, customized Market Access Strategy that acknowledges **country-specific insights**
- Especially **small to mid-size biotech companies** need to develop country-by-country strategies with **local consulting experts** that are supervised under a central approach

For additional information or to request a copy of this presentation,

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