ASSESSING THE EVOLUTION OF THE PRICING AND ACCESS LANDSCAPE FOR CELL AND GENE THERAPIES IN THE US AND EU3 THROUGH A WEB-BASED PORTAL TO ENGAGE PAYERS

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MAT|RPR RAPID PAYER RESPONSE

INTRODUCTION

Cell and gene therapies (C&GTs) is a new concept of transformative therapies, administered once in a lifetime. The C&GTs in this research denotes both cell therapy and gene therapy used separately or together as one therapy. These therapies hold great promise to provide novel treatment modalities for a wide range of severe diseases, especially in rare diseases and oncology. However these therapies are generally costly, partly as a result of the high cost of development, manufacturing, and clinical administration, and potential of long-term efficacy. There are limited studies in literature which focuses on the impact of these challenges on reimbursement decision making from payer's perspective. The current research seeks to understand payer perceptions on the reimbursement challenges, expected evolution of policies and potential payment mechanisms in relation to C&GTs.

METHODOLOGY

A web-based survey was administered through the Rapid Payer Response™ online portal (RPR®) to 25 payers with experience in HTA and reimbursement decision-making in the US (10 payers), France, Germany, and the UK, 5 payers per country. Payer profiles included Commercial & Medicare payers in US, former members of NICE (The National Institute for Health and Care Excellence) and the SMC (Scottish Medicine Consortium) in the UK, ex-CEPS and ex-TC (Transparency Committee) payers in France, ex-GBA (Federal Joint Committee) and SHI (Social Health Insurance) payers in Germany. Responses were collected through the RPR® interactive platform in 5 days allowing opportunity to ask clarifying and follow up questions to triangulate insights.

OBJECTIVES

The specific objectives of this survey are:

- To evaluate the impact of current market access challenges on reimbursement decision making of C&GTs
- Understand the current and future reimbursement approaches for C&GTs
- Gain insight on preferred payment mechanisms and their likelihood of use for management of C&GT therapies funding and access

RESULTS

Landscape of market access for cell and gene therapies

Early C&GT entrants achieved variable reimbursement and encountered significant access barriers. In US, Provenge® faced a challenge of delay in securing FDA approval and CMS coverage, as well as the high cost that had to be incurred by providers up-front. While Glybera® secured a positive HTA outcome in Germany, access was achieved in only one patient due to a lack of adequate financing mechanisms for costly one-off treatments.

The reimbursement of recently approved C&GTs Kymriah®, Yescarata® and Luxturna® holds great promise for the future therapies. The speed at which these therapies secured reimbursement in some markets, is driven by significant therapeutic improvements they bring, but also indicates better payer preparedness for assessment and access negotiations for C&GTs. However, there are commercialization challenges particularly focused on payment models which remain to be addressed.

C&GTs	EU	US	US	France	Germany	UK***
Chondrocelect®*	Oct 2009	-		SMR (IV)	Not eligible for EBA	
Glybera®*	Oct 2012	-		SMR (IV)	Non-quantifiable	
MACI®*	Jun 2013	Dec 2016	Covered		Not eligible for EBA	Recommended
Provenge®*	Sep 2013	Apr 2010	Covered		Non-quantifiable	Non Recommended
Holoclar®	Feb 2015	-		SMR (I), ASMR (IV)	Not eligible for EBA	Recommended with restrictions
Imlygic®	Dec 2015	Oct 2015	Covered		No added benefit	Recommended with restrictions
Strimvells®	May 2016	-				Recommended (HTS)
Zalmoxis®**	Aug 2016	-		SMR (IV)	Non-quantifiable	
Spherox®	Jul 2017	-				Recommended
Alofisel®	Mar 2018	-		SMR (I), ASMR (IV)	Non-quantifiable	Not Recommended
Kymriah®	Aug 2018	Aug 2017	Covered	DLBCL: SMR (I)/ASMR (IV) b-ALL: SMR (I)/ASMR(III)	Non-quantifiable	Recommended (CDF)
Yescarta®	Aug 2018	Oct 2017	Covered	DLBCL: SMR (I)/ASMR (III)	Non-quantifiable	Recommended (CDF)
Luxurna®	Nov 2018	Dec 2017	Covered	SMR (I), ASMR (II)	Ongoing assessment	Recommended (HST)

Table 1. HTA decisions for C&GTs approved in US and EU3

* Product is withdrawn from the market (EMA). ** EMA approval revoked. ***For UK, NICE assessment is considered
ALL, Acute Lymphocytic Leukemia; CDF, Cancer Drugs Fund; EBA, Early Benefit Assessment; DLBCL, Diffuse large B-cell lymphoma; HST, Highly specialised technology

Positive assessment/Uncertain benefit | Restricted reimbursement | Negative assessment | Not assessed

Access challenges for C&GTs

Overall, uncertainty around the evidence at launch is the highest rated challenge reported by the decision makers. The lack of adequate payment mechanisms is also noted as a challenge, especially by payers in the US and France. Logistical set up of centers of excellence and ensuring adequate patient access is recognized only as a moderate challenge that can be overcome with adequate planning and collaboration among stakeholders.

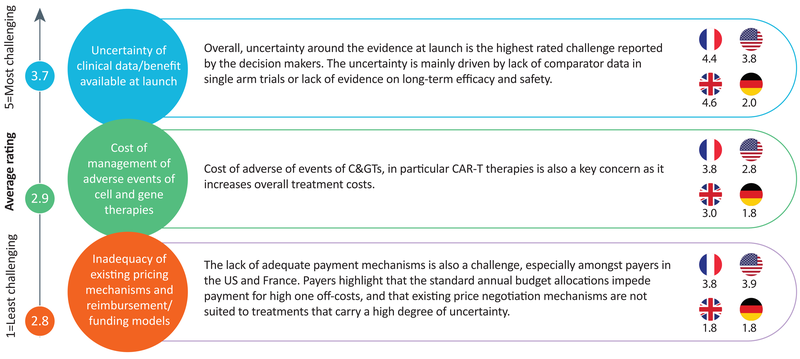

Figure 1. Top three market access challenges for C&GTs from the payer perspective across the scope countries

New policies or changes in funding impacting access to cell and gene therapies

In response to the complex challenges encountered in reimbursement and access of C&GTs so far, several policy and funding changes are considered that may be implemented in the near future. Payers highlight that the solutions need to be multi-fold with collaboration between governments, payers, manufacturers, non-profits, and other stakeholders.

Country	Future policy and funding changes impacting access to C&GTs
France	• DRG add-on payment for administration of C&GTs • Collection of data for registries is also on the horizon for management of C&GTs
Germany	• The proposed risk structure compensation pool law would lower financial risk for payers and lower the barriers to access to C&GTs • The GSAV law (passed on 28th June 2019) introduced new reassessment rules for orphan drugs which will impact some C&GTs
UK	• There are no specific policies for C&GTs in the near future, however oncology C&GTs will continue to gain reimbursement through the Cancer Drugs Fund
US	• DRG add-on payments will change to reflect higher costs of administering C&GTs

Table 2. Potential future policy and funding changes impacting access to C&GTs

Preferred funding/ payment schemes for C&GTs

Coverage with evidence of development (CED) is viewed as the most applicable payment mechanism for C&GTs. CED is already utilized for CAR-T coverage in the UK through the Cancer Drugs Fund and payers in other scope markets consider similar mechanism to be suitable when supported by improving data systems and registries. Still, many payers consider that discounting, payment for performance and capping will continue to be key in facilitating access to C&GTs. Most payers remain sceptical of the feasibility payment by instalments funding mechanisms, apart from US and Germany where respondents expect a combination of tools will be key to facilitating access to C&GTs.

Payment machanisms	Overall	France	Germany	UK	US	Likehood of use for C&GTs in the next 3 years (Very low(1) – Very high(5))
Coverage with evidence of development	4.0	4.0	3.6	4.4	4.0	4.8
Discounting	4.0	3.0	4.4	4.4	4.0	3.8
Pay for performance	3.5	4.0	3.0	3.0	4.0	4.0
Capping	3.3	4.4	4.0	1.6	3.3	3.5
Re-insurance	3.3	3.0	4.4	1.6	4.0	2.5
Price volume agreements	3.0	3.0	3.0	1.8	4.0	3.5
Payment in instalments	2.9	1.8	4.0	1.6	4.0	2.8
Expended risk pools	2.9	1.6	4.4	1.6	4.0	2.0
Supplier credit	2.4	3.0	1.6	1.6	3.3	2.0

High impact | Moderate impact | Low impact

CONCLUSIONS

As payers prepare for approvals of further C&GTs several policy changes are likely to be implemented that will widen innovative contracting opportunities and thus facilitate access but may also limit pricing. Early engagement with stakeholders and preparedness for outcomes-based risk sharing or coverage with evidence development agreements would facilitate speed to market. The ability to anticipate payer behaviour and navigate an increasingly complex access pathway will be critical for achieving optimal access for C&GTs.

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