STEM CELL THERAPEUTICS: THE NEED FOR EARLY MARKET ACCESS

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BACKGROUND/INTRODUCTION

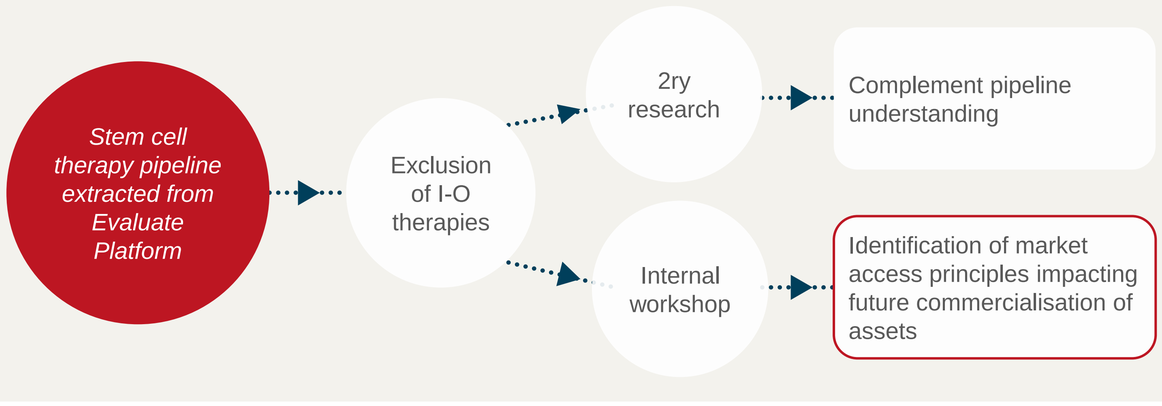
- Significant growth is anticipated in the cell and gene therapy industry, with an estimated 75 therapies to be approved in the US by 2030[1]
- As of December 2023, the global stem cell therapy market was valued at $286 million, with projections indicating a potential increase to $615 million by 2028[2].
- This market falls under the regenerative medicine category, and has the objective of repair, replace, or regenerate damaged tissues and organs to improve patients' lives.
- The main sources of stem cells for regenerative medicine include embryonic, foetal, adult and induced pluripotent stem cells (derived from differentiated somatic cells that have been genetically modified)[3].

OBJECTIVE(S)

- We aimed to better understand the regenerative stem cell therapeutics market and identify the market access pain point manufacturers can face in the commercialisation of these type of therapeutics

METHODS

- An initial identification of the stem cell therapeutics pipeline was download from Evaluate platform®
- Immune-oncology assets were excluded from the initial pipeline to capture only regenerative Stem Cell Therapeutics.
- An in depth review of each asset in the pipeline was conducted to define:
  - Cell Source
  - Therapeutic Area
  - Type of therapy (autologous or allogeneic)
  - Company headquarter
- Internal workshops were used to identify market access principles impact in stem cell therapeutics commercialisation



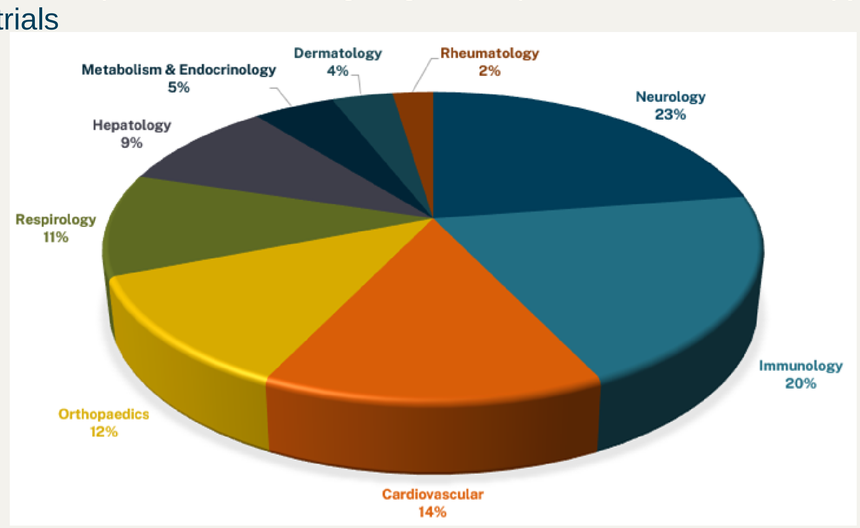
DISCUSSION AND CONCLUSIONS

- Stem cell therapies pipeline is very heterogenic, stem cell therapies can be autologous (using the patient's own cells) or allogeneic (using donor cells) and target various diseases across different stages of development.
- Allogeneic stem cell therapies are dominant in the clinical trial pipeline, probably due to lower manufacturing costs compared to autologous therapies.
- Marketed stem cell therapies target rare diseases and haven't achieved broad reimbursement, albeit targeting niche indications with high unmet need and perceived high willingness to pay (**Table 1**).
- Market access planning for regenerative stem cell therapeutics is similar to other Advanced Therapeutic Medicinal Products (ATMPs), with early planning being critical for commercial success of the asset (**Table 2**).
- As for all ATMPs, consideration on the impact of European joint Clinical Assessment (JCA) and it's impact on market access process and timelines.
- Many of the principles presented in this poster can also apply to other ATMPs, especially cell therapies.

RESULTS

- There is a diverse pipeline with over 200 assets which span more than 10 therapeutics areas. The top 3 areas are Neurology, Immunology and Cardiovascular (**Figure 1**).

Figure 1 Therapeutic areas being targeted in phase 2 stem cell therapy clinical trials



- Adult and foetal stem cells are the most common sources of cells used in therapeutics, likely driven by their low risk of tumorigenicity compared to the other stem cell sources
- 76% of stem cell therapeutic assets correspond to allogeneic therapies. Allogeneic therapeutics can benefit from economies of scale and lower manufacturing costs compared with autologous therapeutics.
- US, South Korea, China are the top 3 countries where companies developing stem cell therapeutics are headquartered. Collectively these countries are responsible for 52% of phase 2 clinical trials in these market.
- Only 3 marketed non-graft stem cell therapeutics are being commercialise, their details are presented in **Table 1**
- Based on the pipeline, manufacturers will face a host of challenges, to which market access principles can be applied to mitigate risk (**Table 2**)

Table 1 Details of marketed stem cell products (non-graft only)

Product	Approval entity	Indication	Line of treatment	Reimbursed	Prevalence	Price	Sales
Neuronata-R	KFDS (South Korea)	Delay ALS progression	Not defined	No	~19.7/100,000[4] (Rare disease)	U$55,000[5]	>U$16.5 M since launch in 2015[6]
Holoclar	EMA (EU)	Severe eye damage (limbal stem cell deficiency)	Last line	Partial[9]	~3/100,000 (Ultra – rare)[7]	$92,000[8]	N/A (privately held company)
Alofisel	EMA (EU)	Complex anal fistula in Chron's disease patients	Last Line	Partial[9]	~7.6/100,000 (Rare disease)[11]	U$55,000 – 65,000 [8]	U$3.5M Jun '21 sales[10]

Notes: Neuronata-R sales were calculated based on over 300 patients since launch (private market)[6].

Table 2 Overview of market access challenges associated with stem cell therapies

Stem Cell Therapies		
Access Challenge	Similarities vs Gene Therapies	Differences vs Gene Therapies
(1) Indication Targeting	Need to select an indication with an unmet need	More potential target indications (SCT can target any cell in the body)
	Need to select an indication that has commercial value	Choice of indication from a long list is crucial to funding (Large proportion of early-stage biotech)
(2) Asset value considerations	Same approach to assess value	Greater ease of scalability, resulting in reduced price expectations from payers
	One-time, long-lasting treatments (autologous stem cells)	Allogeneic SC products – potentially to be administered more frequently (than one-time)
(3) Asset pricing	Market specific pricing process based on value assessment	Lower price expectation from payers (allogeneic therapies have greater economies of scale)
	Public and private healthcare settings considered for launch	No solid benchmarks for pricing stem cell therapies
(4) Lifecycle management of the asset	Lifecycle management strategy and sequencing need to be considered prior to commercialisation	Process patents instead of product patents
		Higher relevance of indication sequence
(5) Evidence requirements	Similar evidence requirements	No differences identified
	Uncertainties in duration of effect	

Abbreviations: SCT: stem cell therapy.

RECOMMENDATIONS FOR MANUFACTURERS

Assess the market early

- Consider market landscape for target indication, including unmet medical need, launch sequence and alignment with reimbursement policies, from an early stage.

Be clear on price and value

- Pricing stem cell therapies is complex, and manufacturers need to consider various factors like value frameworks, comparators, and payer perspectives.
- Consider forecast and early economic modelling, especially for European markets.
- Validate pricing hypothesis with payer research to strengthen valuations.

Further details in Cogentia whitepaper "Stem Cell Therapeutics: The Need for Early Market Access"

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