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STATE OF THE ART AND FUTURE PERSPECTIVES OF ADVANCED THERAPY MEDICINAL PRODUCTS IN ITALY AND IN THE INTERNATIONAL ARENA

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OBJECTIVES

To draw the scenario of Advanced Therapy Medicinal Products (ATMPs), state of the art and future perspectives, comparing European Union (EU) and United States (US) and to extrapolate the role of Italy in terms of expertise and centres of excellence.

METHODS

Horizon scanning has been performed by analysing different sources: I) EU, US and Italian Official Journals (OJ); II) the European Medicines Agency (EMA), the Food and Drug Administration (FDA) and the Italian Medicines Agency (AIFA) websites; III) clinicaltrials.gov.

	1997	1998 - 2008	2009	2010	2011	2012	2013	2014	2015	2016	2017
EMA	0	0	1	0	0	1	2	0	2	2	1
FDA	1	0	0	1	2	3	2	0	1	4	2

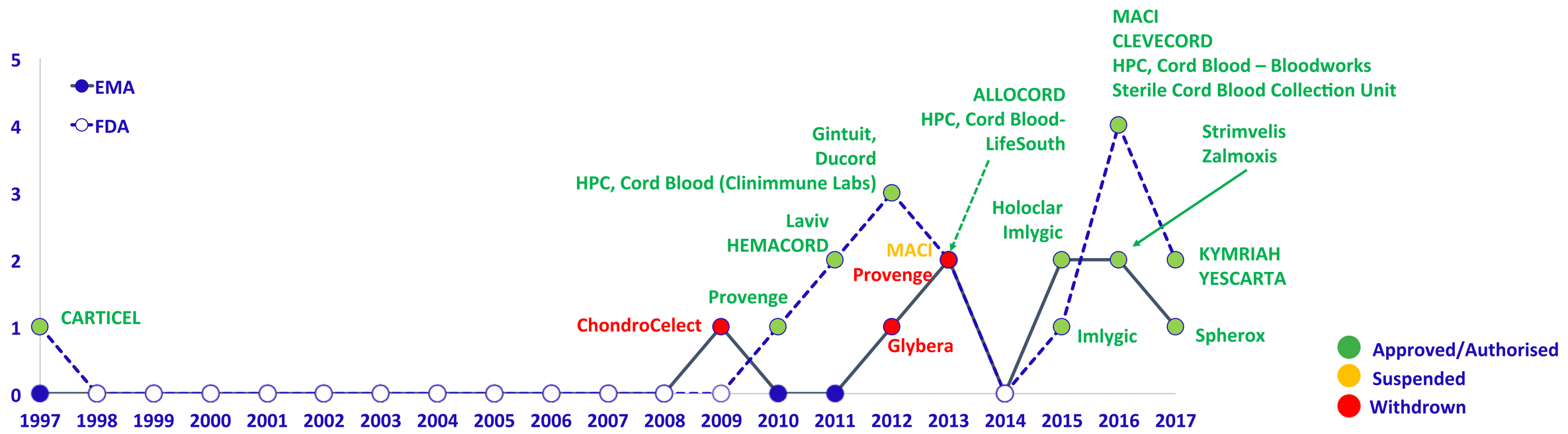

FIGURE 1. ATMPs approved by year (EMA vs. FDA)

Source	ClinicalTrials.gov
Time of analysis	June 2016 – November 2017
Key words	From Hanna et al. 2016
Study type	interventional
Phase	III, II/III
Status	On-going or concluded
TOT. Clinical trials	**135**

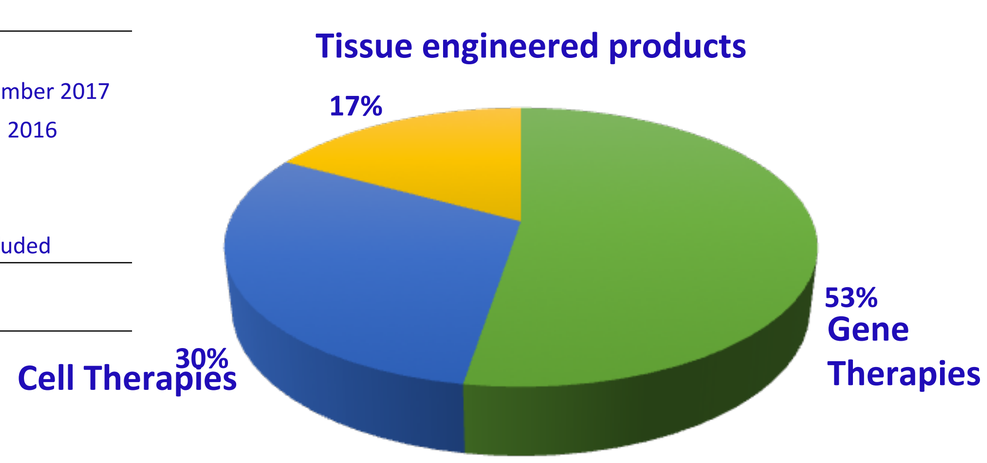

FIGURE 2. Phase III and II/III clinical trials on ATMPs from June 2016 to November 2017

FIGURE 3. 15 cell factories have been accredited by AIFA for ATMPs manufacturing

RESULTS

Results can be divided into 3 parts: i) state of the art; ii) future perspectives and iii) Italian context. i)The ATMPs scenario is quite different in EU and the US: the definition of ATMPs is not univocal, but differs between EMA and FDA. Specifically, 9 ATMPs have been approved by EMA vs 16 approved by FDA, of which cord blood products (n=8) are considered ATMPs by FDA only. Only 3 ATMPs have been approved both by EMA and FDA. ii)Clinical trials screening has identified 135 Phase III and Phase II/III trials from June 2016 to November 2017, 22 of which (16%) have been already completed. Only 19% of these RCTs are on rare diseases. Furthermore, 5 ATMPs are currently under EMA evaluation. iii) Reimbursed ATMPs in EU have been developed and produced in Italy. Furthermore, 53 Italian centres have been involved in clinical trials on ATMPs and 15 cell factories have been set.

CONCLUSIONS

ATMPs number is expected to grow significantly in the very next future in EU as well as in the US. In this context, Italy can be play an interesting role for clinical expertise and for Italian experience on price and reimbursement process.