



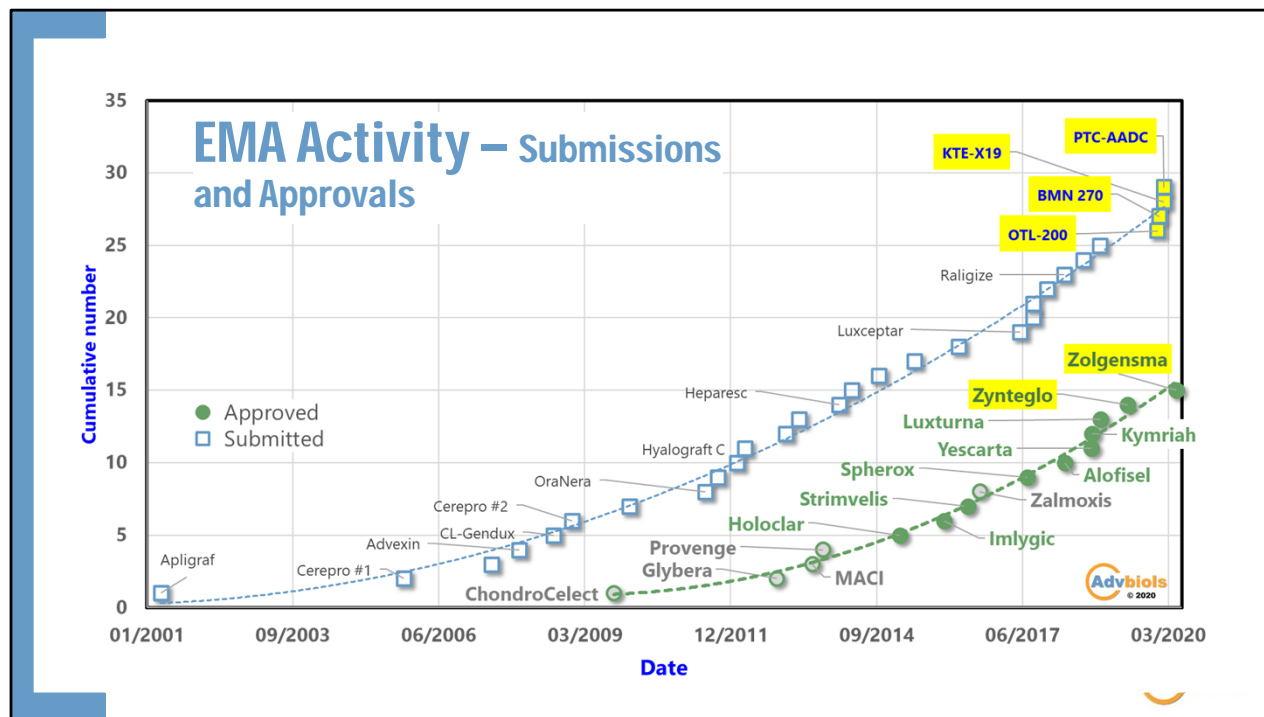
**Chair Introduction**  
**Cell Therapy CMC and Manufacturing**  
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This time last year Zynteglo had just been given a conditional approval (May) for beta thalassaemia, an orphan indication affecting 1:10,000 of the population. The first ATMP to complete the procedure under accelerated assessment, others haven't managed to stay on that timetable.

Rarer still at 0.4 in 10,000, and after some issues with data integrity, Zolgensma was also given a conditional approval in May this year.

Unfortunately these two gains are off-set by another loss as MolMed withdrew the conditionally approved Zalmoxis when an interim analysis of the ongoing pivotal study didn't confirm efficacy. This is a sobering example of the risk of conditional approval.

However, there are 4 reasons to be optimistic that further approvals will come this year;

KTE-X19 – Kite, CAR-T for mantle cell lymphoma

OTL-200 – GM-CD34 expressing arylsulfatase to treat metachromatic leukodystrophy (MLD)

BMN 270 – BioMarin, AAV5 for severe haemophilia A.

PTC-AADC – PTC therapeutics Intl, AAV5 for aromatic L-amino acid decarboxylase deficiency.



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**MUCH MORE TO COME**

