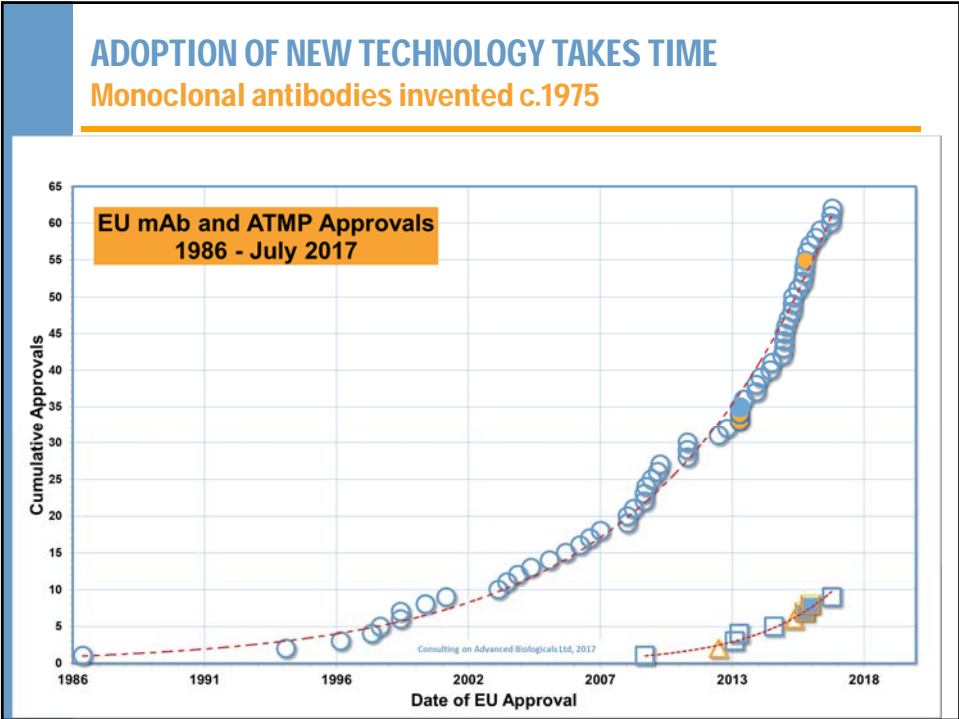


Consulting on Advanced Biologicals

Chair Intro

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EMA Experience To 2017

Gene Therapy Medicinal Product										Cell-based Medicinal Products										Orphan Opinion MAA
W	W	W	W	N	P	P	P	N	P	P	P	P	P	W	W	P				
W	W	W	W	E	F	F	C		C	F	F	F	F			F				
Cerepro Ark Therapeutics	Advexin Gendux Molecular Limited	Contusogene ladenovec Gendux Molecular Limited	Cerepro Ark Therapeutics	Glybera uniQure-biopharma B.V.	Imlygic Amgen Europe B.V.	Strimvelis GlaxoSmithKline	Zalmonoxis MolMed S.p.A.	Heparesc Cytomet GmbH&Co KG	Holoclar Chiesi Farmaceutici S.p.A.	Spherox CO.DON AG	MACT Verheul-Denmark-AppS	Provenge Dendreon UK Ltd	Hyalograft C Anika Therapeutics S.r.l.	OraNera CellSeed Europe Ltd	ChondroCelect (2007)					
Ad5	Ad5	Ad5	Ad5	AAV	HSV	RV	RV		Auto	Auto	Auto	Auto	Auto	Auto	Auto	Donor Vector				



MORE ON THE WAY? EMA APPROVALS in 2017

- ▶ Tigenix – Positive Dec 2017
- ▶ Cx601 (eASC)
 - ▶ Anal fistulas, Crohn's disease



MORE ON THE WAY?

EMA On-Going Assessments

- ▶ **Kiadis – Submitted May 2016**
 - ▶ ATIR
 - ▶ Haploidentical donor lymphocytes depleted of alloreactive T cells
 - ▶ for the treatment of prevention (reduction) of transplant-related mortality (caused by graft-versus-host disease and/or infections) following haploidentical allogeneic hematopoietic stem cell
- ▶ **Spark Therapeutics – Submitted July 2017**
 - ▶ voretigene neparvovec (LUXTURNA™)
 - ▶ AAV2-hRPE65v2
 - ▶ vision loss due to Leber congenital amaurosis or retinitis pigmentosa caused by confirmed biallelic RPE65 mutations.



MORE ON THE WAY?

EMA On-Going Assessments

- ▶ **Kite Pharma – Submitted July 2017**
 - ▶ axicabtagene ciloleucel (KTE-C19)
 - ▶ retroviral vector
 - ▶ relapsed or refractory DLBCL, PMBCL and TFL
- ▶ **Novartis – Submitted Nov 2017**
 - ▶ tisagenlecleucel-T (CTL019)
 - ▶ relapsed and refractory (r/r) pediatric and young adult patients with B-cell acute lymphoblastic leukemia (ALL)



To Consider During Discussions

Have we as an industry been too focussed on regulatory and not enough on commercialization?

- Regulatory approval is a must, but not sufficient;
- Needs to be cost-effective also.
 - CoGs – are we focussing enough?
 - Indication - are we choosing the right indication/s?
 - Are we so 'dazzled' by the promise/potential to make a difference and forgetting it cannot be at **any** price.

