Consulting on Advanced Biologicals

Chair Intro

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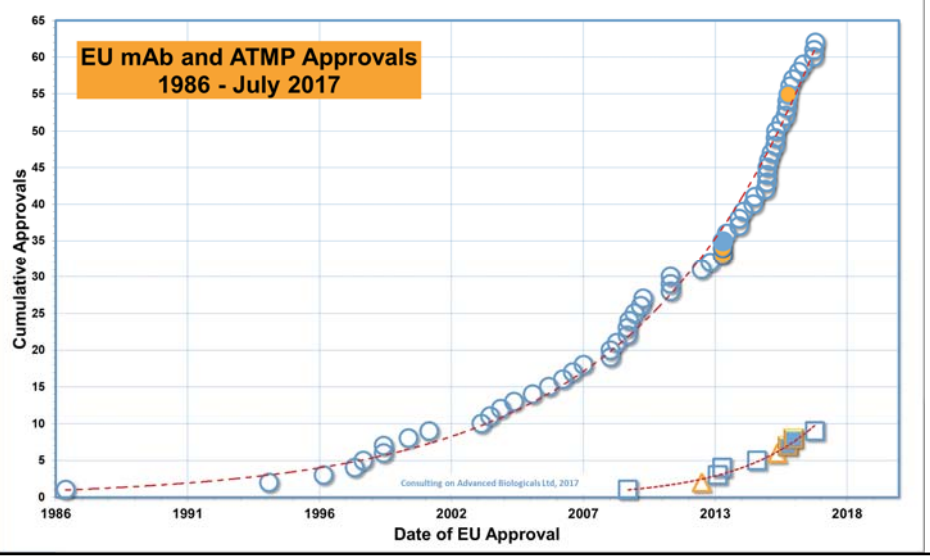
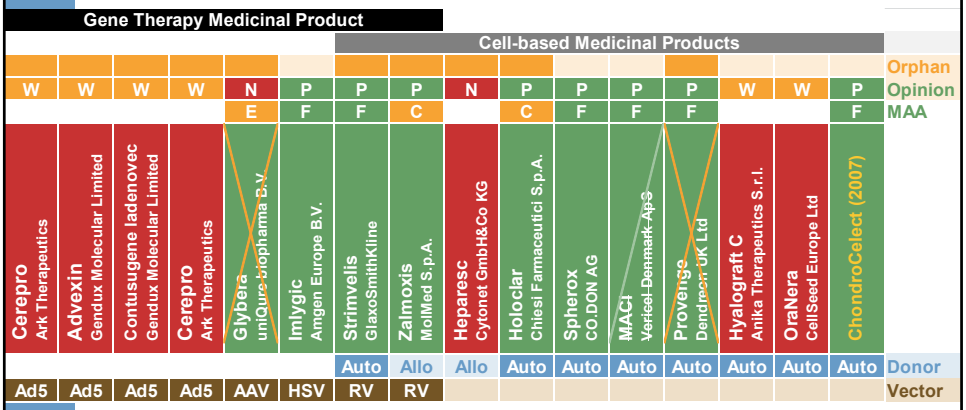
ADOPTION OF NEW TECHNOLOGY TAKES TIME

Monoclonal antibodies invented c.1975



EMA Experience
To 2017



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MORE ON THE WAY?
EMA APPROVALS in 2017

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

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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 transplantrelated mortality (caused by graft-versushost 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.

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