Pathways in the regulation of clinical stem cell research: harmonization, “double discourse” and alter-standardization

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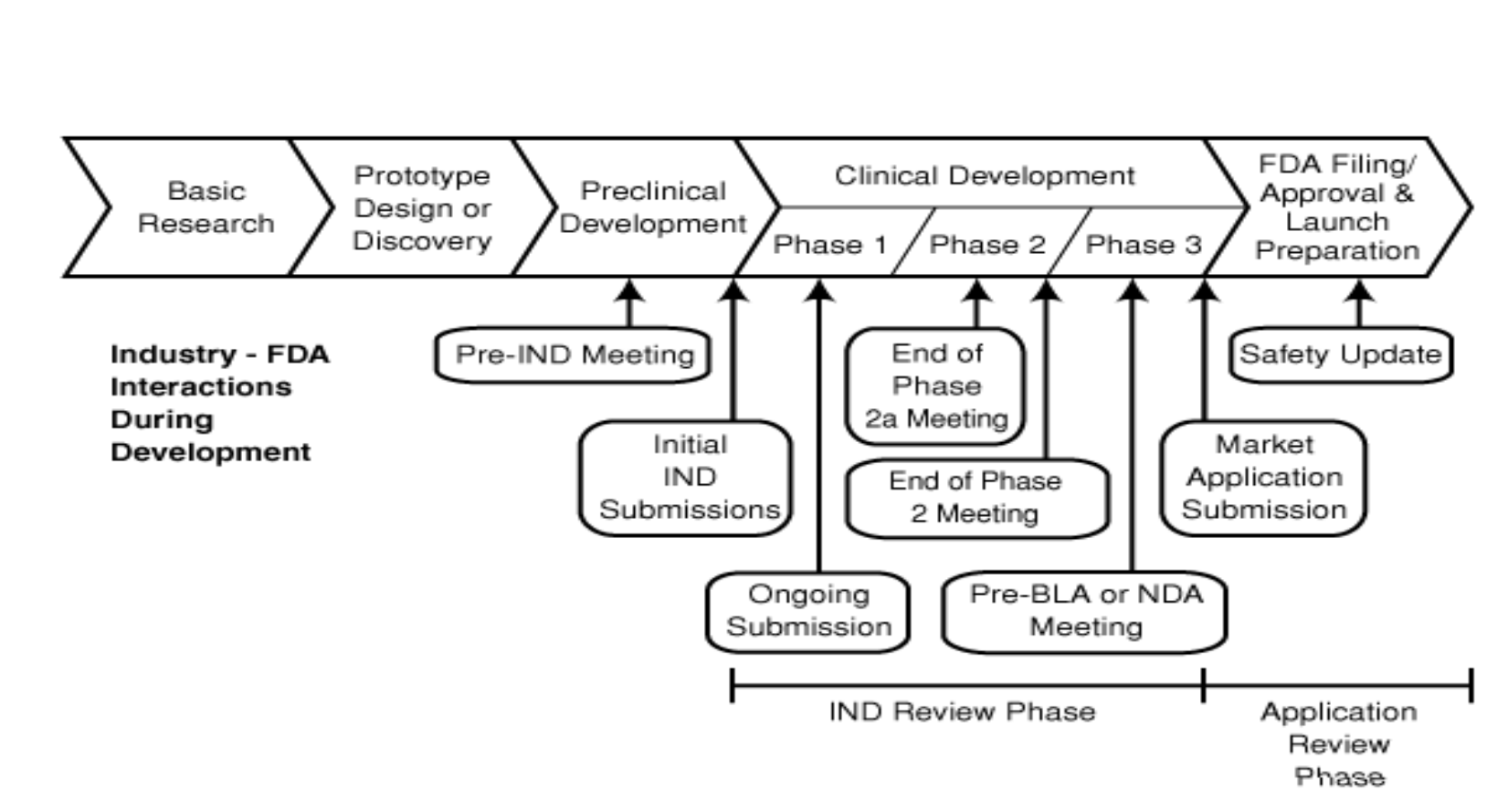
Research questions

- What are the current trends and developments in the regulation of clinical stem cell interventions?
- In which ways do these developments challenge the dominant paradigm of Evidence Based Medicine and multiphase randomized clinical trials in stem cell research?

The standard model for pharmaceutical development



Source: FDA

Specificities of stem cell clinical applications

- Ambivalence between “product” and “procedure”
- More small-scale practices possible
- Personalized treatments

- More possibilities of innovation and empowerment of local physicians
- But… makes the generation of reliable data on safety and efficiency harder and increases the possibility of “scams”.

Orthodox pathway: harmonization

- Cluster EMA-FDA-Health Canada
- Harmonization of legislation under the concept of "Advanced Therapy Medical Products" (ATMP)
- SC interventions must prove safety and efficacy through traditional EBM standards.
- Systems however allow for nuances, exceptions and exemptions:
  - Difference between "minimally manipulated" and "more than minimally manipulated"
  - Hospital exemption (EU)
  - Compassionate use/expanded Access
  - Fast track/accelerated or conditional approvals

“Double discourse” pathway

- Exemplified by India and China.
- Formal legislation is compliant with the EBM model but not systematically enforced.
- Legal loopholes and ambiguities in legislation
- “Double discourse” serves to the fulfillment of interests of different stakeholders:
  - Formal legislation addresses the concerns of corporations and elite scientists, both local and international.
  - Tolerance and non-enforcement serve the interests of small scale local researchers and companies.

Alter-standardization pathway

- A divergent model from EBM is explicitly supported by legislation.
- Japan's Regenerative Medicine Promotion Act (2013) and amended Pharmaceutical Affairs Law (2014)
- Conditional, limited-term approval of SC products
  - Granted after first-in-human studies show safety and "likely predict efficacy"
  - Given a 7 year period to collect efficacy data (post-marketing) for definitive approval.
  - 70% of treatment costs are reimbursed by the health insurance system.

Conclusions

- The regulatory landscape is undergoing a process of diversification
- EBM paradigm is losing hegemonic status.
- Even in the countries following the "orthodox" pathway, the number of exceptions and flexibilization options is growing.
- Japan's initiative represents a bold move from an ICH-member.
- Tensions between different models of wealth creation in biomedicine are expressed in regulatory arrangements (Sleeboom-Faulkner).
- Questions about methodological soundness, standards of evidence and ethical acceptability need to be framed and discussed in this new socio-economical landscape.

Thanks for your attention!

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