Lessons Learned from Pediatric Drug Development For the Older Adult Community

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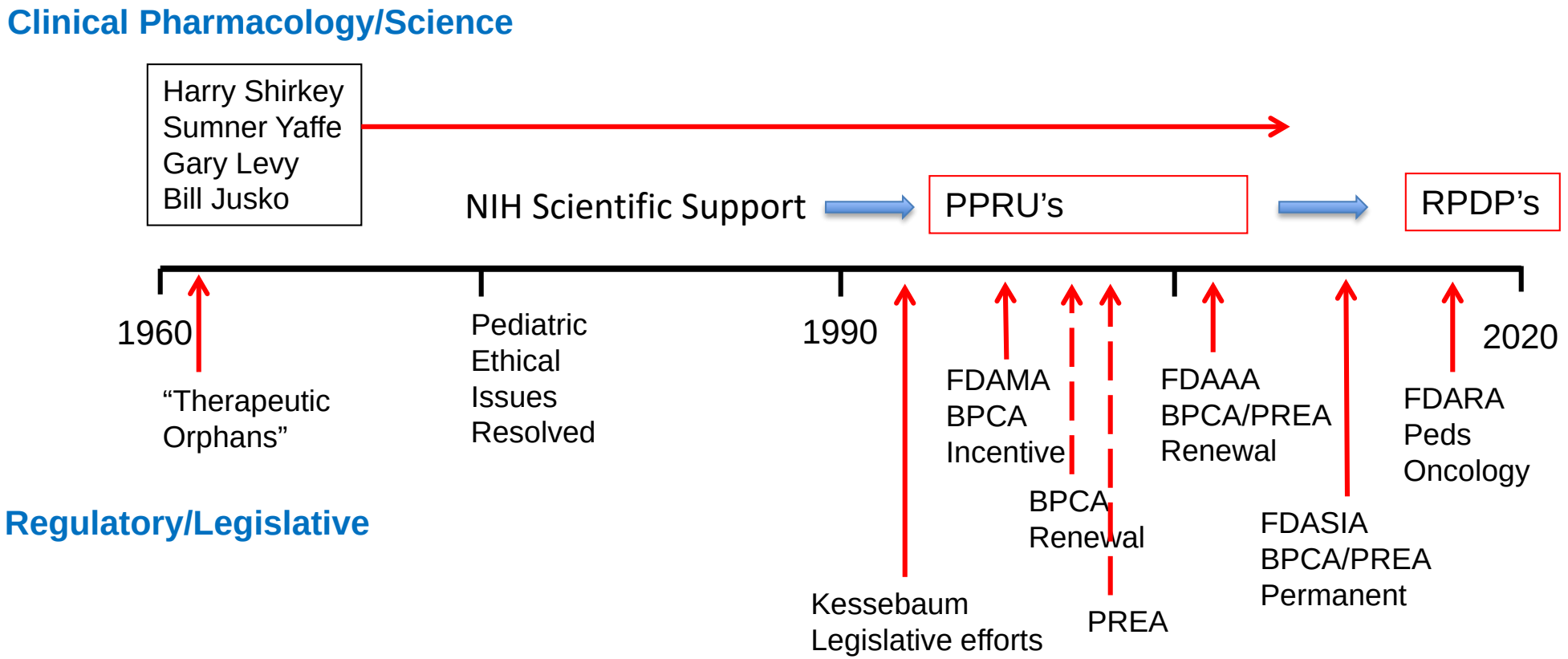
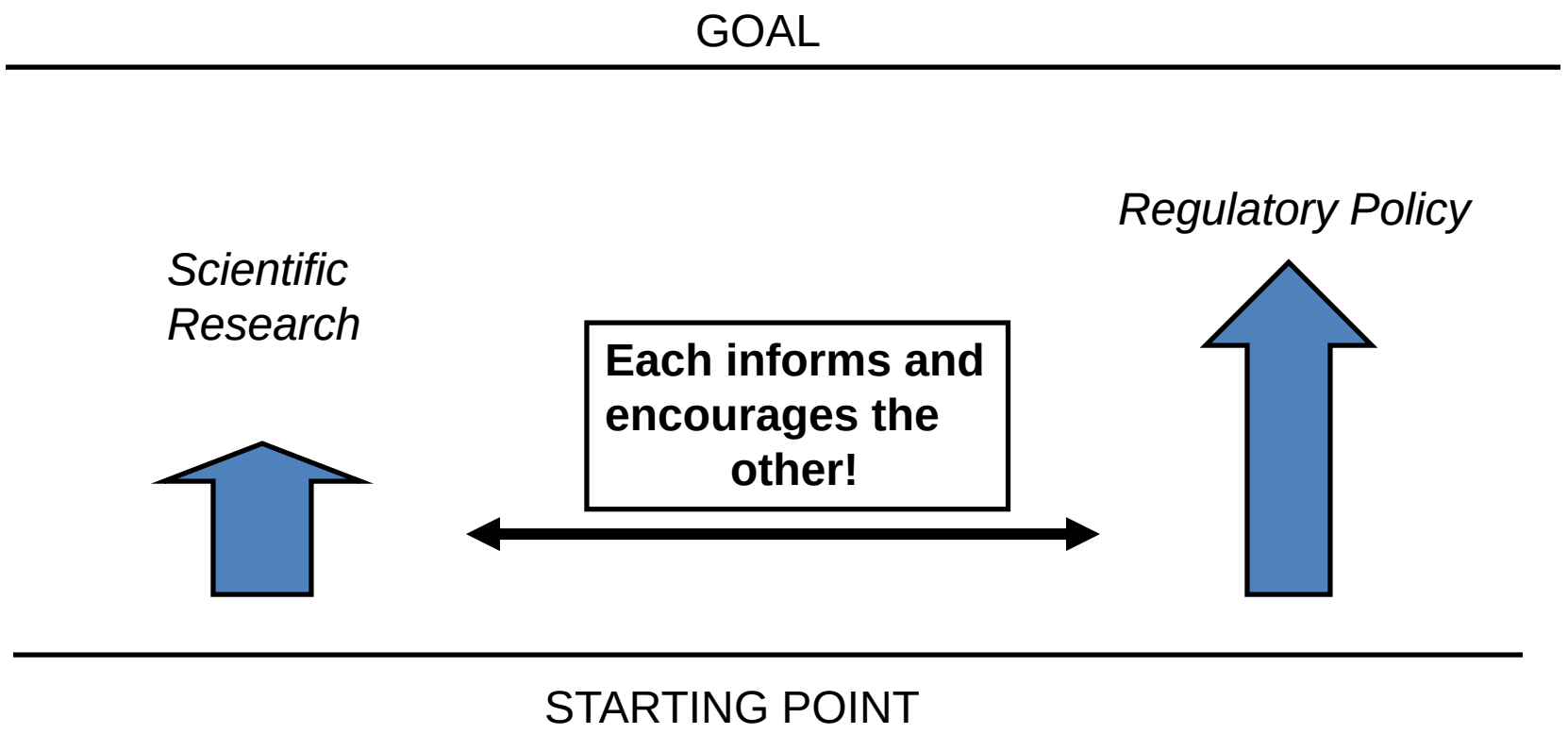
Timeline for Pediatric Drug Development



PPRU's, Pediatric Pharmacology Research Units; RPDP's, Research in Pediatric Developmental Pharmacology; FDAMA, FDA Modernization Act; FDAAA, FDA Amendments Act; FDASIA, FDA Safety and Innovation Act; FDARA, FDA Reauthorization Act; BPCA, Best Pharmaceuticals for Children Act; PREA, Pediatric Research Equity Act

Pediatric model for advancing regulatory science and drug development



Lesson #1: Scientific Advances are Essential

- What is unknown is substantial, so develop a plan that prioritizes the work to be done;
- Recruit partners that provide both the drive and the money to get this done;
  - NIH involvement is essential; while the accomplishments of the PPRU's were not outstanding, they provided a central group of scientists that kept the discussion current and progressive.
- Consider the broader picture of the science that needs to be done: basic science, clinical pharmacology, regulatory science.

Lesson #2: Learn from what did NOT work

- Resolving ethical issues alone did not provide the incentive to get pediatric drug development studies going;
- FDA requests for the voluntary submission of pediatric data for relabeling by sponsors did not produce anything substantial;
- Getting legislation in place is a very slow process.

Lesson #3: Build on what works!

- Pediatric extrapolation of efficacy from the adult patient population works and has expanded;
  - Came from a 1994 FDA pediatric labeling Rule;
- Model Informed Drug Development (MIDD) has made major contributions to advance pediatric drug development:
  - Expanded indications to small groups of patients who would not otherwise have been included;
  - Expedites moving from one group of patients within the “pediatric” sphere to another group without unnecessary studies.

Summary

- The major lessons from the history and progression of pediatric drug development were learned over a painstaking 60 years;
  - Make sure that the science is there to support decisions in drug development for older adults, and develop a plan to advance that scientific knowledge;
  - Pay attention to what has failed in previous specific patient population studies, so that years are not lost on activities that are unlikely to work;
  - Build on what works, especially (a) rules, guidelines and guidances that move the field forward, and (b) applying new tools such as MIDD to advance drug development for older adults quickly.