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Using real data from approved drugs to test analysis and design strategies

- Efficacy data from multiple approved products from placebo-controlled studies
 - Real data with real relationships
- Assemble data at a data analysis site
- Allows testing of statistical methods
- Conduct modeling on enlarged datasets
 - Bootstrapped or permuted data sets
- Create and test new study designs



Analysis of existing approved drugs

- Test methodologies for analysis
 - Control of variation
 - Detection of signal
- Test novel approaches to multivariate or multi-domain analyses
- Retest new study designs
- Use modeling of expanded data sets
 - Study drug vs placebo, placebo vs placebo
 - Sensitivity, specificity



Analysis of clinical study data sets for today

- **Aldurazyme (laronidase)** for MPS I - BioMarin and Genzyme
- **Naglazyme (galsulfase)** for MPS VI - BioMarin
- **Elaprase (idursulfase)** for MPS II – Shire



General considerations for the Workshop

- This will be the first analyses, not the last
 - An example of the first level of analyses
- We hope to get input and suggestions for more work
- We will generate lots of “p values” but we are not hypothesis testing
 - We will do that with modeling of studies later
- Suggest new ideas that we can test