

## *Outlines of Meeting 3.26*

### **Features of Children Diseases:**

- Limited sample size;
- Always can be considered as rare diseases or can be subcategorized into rare diseases, making randomization challenging.
- Usually prescribe multiple drugs/ adopt additional therapies ;
- High Likelihood of recurrence (~20%)

### **Implications for drug development and research:**

- Presents challenges in developing new drugs.
- Reliance on external data for analysis.
- Motivates the exploration of **Registry Data**.

### **Common Features of Registry Data:**

- Not collected for clinical trials (distinct from EHR (Electronic Health Record))
- Typically collected by non-profit institutes
- Not regulated or cleaned; lacks preplanning or pre-specified regulations
- May include records of multiple diseases for various purposes within a single database.

### **Main Focuses of Reviewing Registry Data:**

1. Natural History of diseases (What's the progress? Do the diseases cure naturally or through drugs/therapies?)
2. Medicine information

## Next Steps

- Read and understand the Guidelines of FDA: <https://www.fda.gov/media/122425/download>
- Search registry data of the following two diseases:

Diffuse intrinsic pontine glioma (DIPG)

Ewing sarcoma