



**ECD Global Alliance  
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# Importance of Clinical Trials

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# Why Do We Need Clinical Trials

- Clinical trials answer two important questions
  - Does the new treatment work?
  - Is the new treatment safe?

# How Do We Do Clinical Trials

- Clinical trials are usually carried out in “phases”
  - **Phase I:** What is the safe dose?
  - **Phase II:** Does the treatment work?
  - **Phase III:** Is the treatment better than existing options?
- Timeline: 10-15 years

# Clinical Trials: Pros

- **For mankind**
  - Increase in knowledge about particular disease and its therapy
  - Development of new therapies
  - Prove of efficacy or lack of thereof
  - Identification of potential significant side effects
- **For individual patient**
  - Access to new therapies, which are not commercially available
  - Expansion of therapeutic options
  - Standardized protocol-driven therapy
  - Some studies in cancer patients suggested that patients on clinical trials tend to have better outcomes compared to patients treated outside of trials

# Clinical Trials: Cons

- **For mankind**
  - None
- **For individual patient**
  - Need to meet all qualifying criteria, which are usually not flexible
  - Less flexible and often more intense schedule
  - Travel, financial consequences and time commitment
  - Possible risk of unknown/unexpected side effects

# Why Should I Consider Clinical Trials

- Results of clinical trials are important not only for developing new therapies
- Clinical trials can provide necessary evidence to convince payers to reimburse new and effective therapies
- Clinical testing is necessary tool to make the progress happen

# Strategies for Clinical Trials in ECD

- Prognosis and outcomes have dramatically improved; however, overall there is still room for improvement
- We have relatively limited therapeutic armamentarium
- We have limited resources (patients, finances) and large number of questions, which need to be answered

# Strategies for Clinical Trials in ECD

- **Phase I:** Access for ECD patients to these studies, which are often limited to conventional cancers
- **Phase II:**
  - “Basket studies”: clinical trials for patients with any cancer or histiocytosis with certain unifying feature (e.g. vemurafenib in patients with *BRAF* mutation)
  - ECD specific phase II studies: because of limited number of patients this approach should be reserved for promising therapies with high likelihood of FDA/EMA approval
- **Phase III:** not feasible in ECD

# Where Can I Learn About Clinical Trials?

- ECD Global Alliance Website
- [Clinicaltrials.gov](https://clinicaltrials.gov)
- Care Centers

# Examples of Clinical Trials for ECD Patients

- *BRAF* mutation positive
  - **NIH: dabrafenib/trametinib**
  - Multicenter: my pathway (vemurafenib)
  - Multicenter: dabrafenib/trametinib
  - Multicenter: BVD-523
  - Multicenter: PLX8394
- *BRAF* mutation negative
  - Multicenter: BVD-523 (if MAP2K1 mutation present)
  - Multicenter: PLX8394
  - Single Center: everolimus/anakinra

# Take Home Message

Clinical Trials are Part of Standard of Care in ECD