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
- 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