

Global Cancer Clinical Research, Drug Development, and Therapeutic Accessibility Workshop

Paraphrase: How can people around the globe share in advances in cancer treatment?

2018 DUKO MSI Global Workshop

Precision Medicine Landscape

- Clinical Trial Organizations are carrying out high volume umbrella (one indication) and basket (by target) trials, tending towards use of panels and eventually NGS as screening.
- Often involve multiple companies
- Very popular with patients; accrue well
- Not all are definitive
- Some discussion between study designers and regulatory, e.g., FDA, PDMA
- Concerns about availability to population outside major cities
- Example of LLS's AML Master Trial – a trial run by patient advocacy NGO, testing first line therapy therapeutic window trial including novel/novel combinations

Rare Cancer: Registries & Referral

- Patient/provider groups have developed their own registries, some of which have been international, which have contributed to elucidation of natural history and landscape of existing therapy/practice.
 - Example of the PROCLIP project to develop a prognostic index, involving more than just data collection, e.g., central path review, QOL questionnaires.
 - Example of comprehensive collection within a country: French reference networks, e.g., for bone sarcoma; benefit of putting all cases through an MTD and referring to specialty centers: improved diagnosis, surgical outcomes, overall outcomes.
- Potential to match patients with trials; difficulty for patients to find the best trials near them; role of “patient navigators” e.g., examples from advocacy organizations that create custom referral lists based on patient’s medical and social situation.

Global Accessibility

- We are falling short of UN goals for access to anticancer medicines, palliative care meds, and universal health care
- Innovative, highly effective drugs on the WHO EML are not making it to NEMs worldwide, particularly in LMICs
- Out of pocket costs are hitting everyone, but bankrupting patients in LMICs compounding poverty burden
- Access to investigational/new drugs is improved with more critical consideration of clinical trial exclusion criteria and more liberal use of programs like FDA expanded access.
- From patient perspective, areas for improvement included Education (society and medical professionals); 2) Communications; 3) improve health/medical literacy.
- Access to transplants/high-tech care – Entirely absent in many , but working examples where it has succeeded and at relatively low cost.
- Panel discussion: Companies will be risk-adverse regarding eligibility criteria or other modifications that could undermine their studies.

Health Care Coverage Decision Makers

- US:

- CMS process for federal coverage of cost of medications, devices, and diagnostics. Constraints: statutes and decision guidelines including evidence of health outcome improvement, FDA/compendium approval, and for diagnostics, clinical utility; cost is not a part of the coverage determination. NGS recently covered for advance stage cancers.
- Private Insurance – Costs are exceeding employer capacity and being passed to consumer (copays) and limited by need for prior authorization. Possible solution: Outcomes-based pricing, but many barriers to implementation.

- UK

- NICE: Coverage decision based on clinical effectiveness (duration/quality of life) and cost effectiveness (relative to total cost of competing options). NICE gives drug developers increasingly early feedback alongside EMA to help manage risk.

- Australia

- PBAC: Criteria similar to UK NICE plus consideration about patient affordability absent coverage and projected impact on overall government budget. PBAC makes a recommendation to list registered products, and minister can approve. Consumers are responsible for \$40 copay per prescription.

Clinical Research / Access to New Agents

Nearly Developed Economies

Developing Economies

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Next Steps

- How do we distill this meeting for dissemination?
- Combining global efforts to advance availability of care for rare cancers: precision medicine, referral networks, and registries.
- Tough choices: Balancing cancer care availability versus resources globally, a comparative analysis.
- *Ten things you didn't know about global cancer equity (number 8 will shock you!)*