Expanded Access Programs for Drugs and Biologics

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Outline

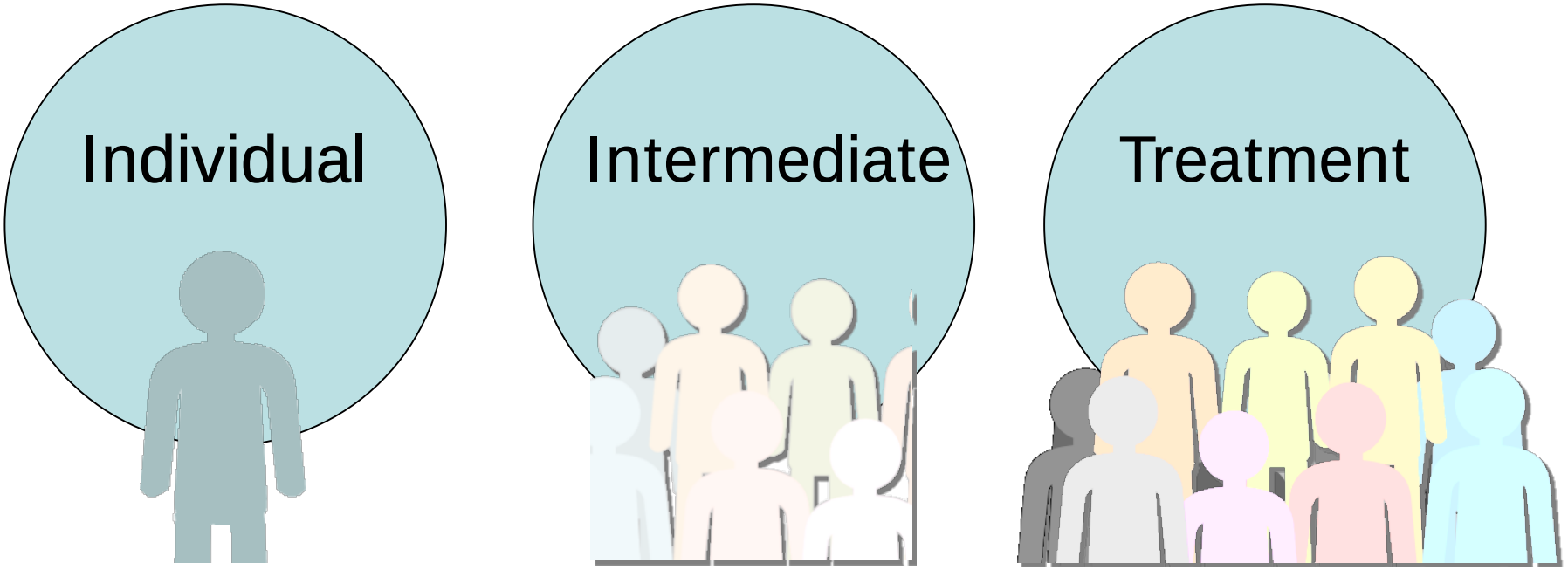
- Expanded Access Programs (EAP)
- Other initiatives to improve access WITHIN clinical trials

Expanded Access

- “Compassionate” use
- You have a serious illness and you’ve tried everything else
- You and your doctor think an investigational drug (not FDA approved) might be a good option
- The drug may be studied in clinical trials, but you are not able participate in these trials

Types of Expanded Access Programs

- 3 types of EAPs are defined in the code of federal regulations (CFR):



Single Patient IND

- Generally patients with multiply relapsed or refractory cancer

- Reasons for requesting expanded access may include:
  - Promising evidence of activity with a drug in a disease with a similar molecular target or histology
  - Patient received benefit while participating on a previous clinical trial
  - Ineligible for clinical trial but reason to think potential benefit outweighs the risk
  - Clinical trial is closed to accrual
  - Drug is not currently being developed
  - Clinical trial site not accessible to patient (regional)

How to Apply for Expanded Access?

Stay tuned: FDA Call Center Pilot

- "Project Facilitate"
- Make the EA process even more efficient
- Telephone number for physicians
  - Resources: independent IRBs
  - Contact information for the drug company
- FDA/OCE to host a public meeting in mid-May

1. Risk has not been established for investigational drug

2. Potential benefit is often overestimated

Pros

- Provides access to potentially lifesaving therapies to patients who have no other alternatives, & may be willing to accept greater risk
- Provides patients a measure of autonomy over their own health care decision
- Bridges gap between drug development and FDA approval
- May provide data to support development
- May offer hope for patients with no other available options

Cons

- Risk has not been established
- May overestimate benefit and underestimate risk
- Drug availability
  - manufacturing
  - fear that adverse events on EAP may disrupt drug development (MYTH!)
- Paperwork! (improved, & ongoing initiatives to overcome)

Could Expanded Access Be Made Obsolete?

- Expanded access programs are in place when no appropriate alternatives exist, but the best access is an approved drug
- To be part of the road to approval, enrollment/treatment on clinical trials is critical

- Considerations for decreasing the need for expanded access in oncology:
  - Expansion of eligibility criteria (broadly)
    - Age, CNS disease, organ dysfunction
  - Separate cohort within a clinical trial with broad eligibility criteria
  - Novel trial designs: Master protocols
    - May allow assessment of multiple diseases, treatments, or biomarkers in one protocol
    - Example: Pediatric MATCH
  - Initiatives in pediatrics: FDARA

- The future: novel surrogate endpoints, real-world data mining, personalized medicine

Summary

- Expanded access programs provide access to unapproved, investigational therapies to patients who have no other alternatives
- The single patient IND is the type of expanded access oncologists would most likely encounter
- The single patient IND requires agreement from the patient and doctor, the drug company, the FDA, and the IRB
- Oncology stakeholders are considering options to try and improve access to unapproved drugs

Resources for Single Patient INDs

- https://www.fda.gov/Drugs/DevelopmentApprovalProcess/HowDrugsareDevelopedandApproved/ApprovalApplications/InvestigationalNewDrugINDApplication/ucm107434.htm

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