Navigating Expanded Access

Patient Advocacy Perspective
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Kidsvcancer.org
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Our programs

• Creating Hope Act
  - Pediatric Priority Review Vouchers
• RACE for Children Act
  - Research to Accelerate Cures and Equity for Children Act
  - Introduced July 2016 to update PREA
• Compassionate Use Navigator
• Post-mortem Brain Tissue Donation
Compassionate Use Navigator

• Created in April 2016. Inspired by Josh Hardy’s case.

• Josh’s case study is available at http://www.kidsvcancer.org/compassionate-use/reports/.
Compassionate Use Navigator goals

• Assist physicians and families (single patient IND).
• Address informational barriers.
• Measure the unmet need.
The need for a Navigator

Survey of pediatric oncologists, N = 74, convenience sample of 500, preliminary results

• **59 out of 74** physicians think that a service assisting with compassionate use applications would be helpful.

<table>
<thead>
<tr>
<th>NEVER APPLIED FOR COMPASSIONATE USE</th>
<th>39</th>
</tr>
</thead>
<tbody>
<tr>
<td>- Not familiar with compassionate use</td>
<td>8</td>
</tr>
<tr>
<td>APPLIED FOR COMPASSIONATE USE</td>
<td>25</td>
</tr>
<tr>
<td>- Clinical benefit seen “sometimes” (~50% of cases)</td>
<td>17</td>
</tr>
<tr>
<td>- Clinical benefit seen “often” (~80% of cases)</td>
<td>7</td>
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Compassionate Use Navigator flowchart

Available on the website, Kidsvcancer.org.
Compassionate Use Navigator services

For Families

• Personal assistance in completing the application;
• Step-by-step instructions;
• Points of contact and expanded access policies at drug companies;
• Information on FDA procedures and IRB approval process;
• Forms and templates, including adverse events reporting.

For Physicians
## Measuring the need for investigational drugs

Compassionate Use Navigator is building a registry of requests and clinical outcomes – self-reported by a family or a physician.

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<tbody>
<tr>
<td>Applications received by FDA (CDER)</td>
<td>1200</td>
<td>940</td>
<td>977</td>
<td>1882</td>
<td>1262</td>
</tr>
<tr>
<td>Applications allowed to proceed by FDA</td>
<td>1199</td>
<td>936</td>
<td>974</td>
<td>1873</td>
<td>1256</td>
</tr>
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Compassionate Use Navigator is building a registry of requests and clinical outcomes – self-reported by a family or a physician.
Outcomes registry – what data is needed?

1. Numbers and outcomes of applications.

2. Numbers of FDA-authorized expanded access requests for pediatric oncology patients.
   - OHOP data for Jan 2012-Dec. 2014: **1,332** INDs (single patient and emergency use)
   - Requests were for **157** unique drugs; CML (28%), breast cancer (12%), ALL (9%).
   - Age was available for n=827; **8%** age 17 or younger.

3. Data on efficacy and toxicity.
Informational barriers – need for transparency


“Notwithstanding the efforts of the pharmaceutical industry and the FDA, accurate, uniform information for physicians and patients about EAPs is lacking. Which products are available and how to enroll in specific protocols are not well publicized.”
“Certain groups – physicians with clinical research experience, their patients, and computer-savvy patients with time to research EAP drug availability – have a great advantage in gaining access to these limited programs. This is unfair. Patients who do not know about EAP will never have the opportunity to consider it as an option.”

http://jama.jamanetwork.com/article.aspx?articleID=2442925
How do patients find compassionate use drugs?

- Through their physician
- Internet, including ClinicalTrials.gov
- Patient organizations, forums, and word-of-mouth.
How do physicians find investigational drugs?

• Knowledge of clinical trials
• Learn from colleagues
• Drug manufacturers’ websites
• Google?

=> There is a need for an accessible, free, real-time database of drugs in development.
Genomic testing and compassionate use

• Filling the gap between the number of adult cancer drugs and lack of drugs for children

• Problems for physicians:
  1. How to select the drug
  2. How to obtain the drug

• Partnerships with precision medicine teams:
  - Teams address the first problem
  - Compassionate Use Navigator addresses the second problem.
Conclusions

Next steps to improve navigation of expanded access programs:

• Educational efforts for physicians, families, and IRBs;
• Drug identification process or database;
• Company contact database;
• Numbers of requests: by drug, disease, population;
• Outcomes of requests: decisions and clinical outcomes.
Thank you!

• Visit our website: Kidsvcancer.org
• Email: Elena@kidsvcancer.org