Exploring an Expanded Access “Navigator”

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Reagan-Udall Foundation for the FDA (RUF)

- RUF is a non-profit established by Congress
- Goal is to support and advance the mission of FDA
- Serve as an "honest broker" between FDA and external stakeholders
- Tackle complex, multi-sector challenges that require the participation of diverse stakeholders
Single Patient Expanded Access Involves Many Players

- Physician
- Company
- Patient
- FDA
- IRB
Single Patient Expanded Access is a Multi-Step Process

**Steps**

1. Identify Therapy
2. Request EA from Company
   - If yes
     - Request EA from FDA
       - If yes
         - Request Permission from IRB
           - If yes
             - Obtain Therapy

**Key Players**

- **Physician** + **Patient**
- **Physician** + **Company**
- **Physician** + **FDA**
- **Physician** + **IRB**
- **Physician** + **Patient**
Need to Increase Understanding of the Single Patient EA Request Process

- EA is not a primary job of companies, FDA, or IRBs
- Confusion around request process & roles of various players
- No one satisfied with the current process

Complex Landscape
- Social media
- Advocacy
- Press coverage
- FDA Activities
- Industry Activities
- Legislative Activities

Access to **accurate** information seems to be a major barrier
Access to Information is a Barrier

- Few resources dedicated to EA
- Could RUF bring stakeholders together to improve available information
- “Navigator” proposed to fill information AND communication gaps
- Use various approaches to better understand needs
  - Landscape Review
  - Stakeholder mapping
  - Consultations
  - Survey
  - Workshops
  - Public comments

Findings
- Almost no data on administration and utilization of EA
- Information on patient and HCP experience lacking
- Very little physician education
- Low profile from medical professional societies
Challenges in the EA Request Process

Challenges

- Where to look/how to find investigational treatments
- Determining available routes of access: clinical trial, EAP, single patient EA
- How to initiate single patient EA request process

- Locating company information: point of contact, policies
- Paperwork takes time/labor (physician may be hesitant)
- Lack of standardization between companies regarding information provided
- No standard/guaranteed turn-around time

- Locating relevant point of contact
- Paperwork takes time/labor (physician may be hesitant)
- No standard/guaranteed turn-around time
- Understanding reporting requirements
- But, FDA has dedicated resources in the Office of Health and Constituent Affairs (OHCA) and Division of Drug Information (DDI) to provide assistance and information

- Beyond normal IRB purview, EA is not research
- Paperwork takes time/labor (physician may be hesitant)
- Lack of familiarity/accessibility outside academic medical centers
How Could a Navigator Help?

RUF Can Act On
- Promote greater awareness and understanding of request process
- Provide clearer information on how & where to start the EA request process

RUF Can’t Act On
- Company policies
- FDA policies
- Decision-making
- Response times
Proposed Functions

- Health Care Provider Education
- Company Directory

Intended Outcomes

- Increase EA understanding among HCPs and fill gaps in existing information—target those outside research centers
- Increase health equity
- Increase willingness of HCPs to help
- Improve transparency and increase availability of information on company policies and contacts
Providing Educational Content

Education

- Leverage rather than duplicate existing resources
  - Steps in process and roles of each player
  - Eligibility criteria

- Fill gaps, especially for those outside research centers
  - IRB resources
  - Reporting requirements
  - Resources for patient management

- Conduct outreach and communications efforts

- Currently exploring partnerships with medical professional societies for content development and outreach to members

- Success depends on ability to reach healthcare providers

- Determining Functionality
  - Courseware
Providing A Company Directory

Company Directory

- Standardize available information
  - Encourage more companies to share
  - Opportunity for greater harmonization

- Currently exploring partnerships with trade groups for endorsement and outreach to member companies

- Success depends on ability to reach companies and willingness of companies to provide and update requested information

- Determining needed infrastructure and functionality

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Expanded Access Directory

<table>
<thead>
<tr>
<th>Company Name</th>
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<table>
<thead>
<tr>
<th>Expanded Access Policies</th>
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<tbody>
<tr>
<td>Are EA requests considered</td>
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<td>Eligibility Criteria</td>
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<table>
<thead>
<tr>
<th>Point of Contact for EA Requests</th>
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<tbody>
<tr>
<td>Phone</td>
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<td>E-mail</td>
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<table>
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<th>Link to webpage</th>
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What Information Do Companies Provide?

How widely are BIO and PhRMA principles being applied?

Approach

- Informal audit of 29 company websites
  - 22 large cap and 7 small to micro cap
  - Pharma and Biopharma
  - Oncology or Rare Diseases portfolio

- Examined for 4 key pieces of information
  - Point of contact
  - EA eligibility criteria
  - Description of request process
  - Anticipated turn-around time
Results

- No small companies provided **any** info
- Most large companies provided **some** info
A Look at Company Webpages

Of the 78% of large companies providing information:

- Information varied in clarity, depth, and accessibility
- ~50% were “easy” to navigate to from Google
- More difficult within site
Ongoing Activities

- Honing in on Functionality
  - Developing detailed plan
  - Determining funding needs and sources
  - Securing Partners
Thank You