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*Silence your cell phones please*
How to Work with Industry

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What Do We Want or Expect and Ultimately Get?

Partnership Between Biotechnology/Pharmaceutical Companies and a Patient Advocacy Group
Epidermolysis Bullosa - Definition

EB has been called a skin disease (because of its main symptom) and it’s been called a group of disorders (because there are 5 types and 31 subtypes). Yet, neither properly defines the disease.

What is Epidermolysis Bullosa?

Epidermolysis Bullosa (EB) is a rare, genetic connective tissue disorder. There are many genetic and symptomatic variations of EB, but all share the prominent symptom of extremely fragile skin that blisters and tears from minor friction or trauma. Internal organs and bodily systems can also be seriously affected by the disease.

EB is always painful, is often pervasive and debilitating, and is in some cases lethal before the age of 30. The list of secondary complications can be long and may require multiple interventions from a range of medical specialists.

EB affects 1 out of every 20,000 live births and affects both genders and every racial and ethnic background equally. Those born with it are often called ‘Butterfly Children’ because as the analogy goes, their skin is as fragile as the wings of a butterfly.

There is no treatment or cure. Daily wound care, pain management and protective bandaging are the only options available.

By the numbers....

1 out of every 227 people has a defective gene that causes EB
There are about 25,000 people in the US with EB
There are about 30,000 in Europe and 500,000 worldwide
About 200 children are born each year in the US with a form of EB
Partnership with Industry – **What it is NOT**

Let’s just get this out of the way early. A partnership is NOT.....
Partnership – Static or State of Flux?

Who are you partnering with? Is the relationship different depending on stage and type of company?

Does the nature of the relationship change?

YES
Partnership – A Paradigm

Expectations - regardless of who is the partner.

Industry, regardless of whether a startup, an established biotechnology or pharmaceutical company should expect that the

The PAG is the leading KOL

and

We want to be involved early and often

- We know the patients
- We know the disease
- We know the clinicians
- We know the researchers
- We know the burden of illness
- Did I say we know the disease?
Partnership – We Want the Same Thing

It’s important to remember that we are in this together and can help each other.

Don’t demonize industry. We need them to discover, develop, test, and commercialize.

PAG should recognize and appreciate the compliance aspects of development. And how that affects TRANSPARENCY.
What can the PAG expect from industry that defines a successful partnership?

- We want a seat at the table
  - Our input is as valuable as our patient outreach
  - We want transparency – as much as possible
- While one point of contact is good for you it might not be for us
- Programmatic funding
- Awareness – involve us in media/regulator/investor outreach
- Ask what we need from an educational material point of view
- Budget for sponsorship of events/conferences
- Help us fundraise
Registry – An Example of Partnership
Win – Win Situation for All Entities

debra of America

- Quantify the financial, social, physical and financial burden of illness
- Gather prevalence data
- Encourage researchers to mine the data
- Gain further insights into symptoms and overall quality of life issues

New Co.

- Gain prevalence data
- Disease severity
- Pharmaco-economic information
- Natural history data
- Identify patients for clinical trial
- Educate regulators
- Help with reimbursement
Who Owns It?—EB Care LLC
A Third Entity Was Created to Own the Registry

EB Care LLC is controlled by a Board of Managers
• 2 Members from Sponsor
• 2 Members from debra
• 1 International Researcher

Patient Advocacy Group:
• Owns the Data if LLC ceases operations
• Provides Quarterly Reports on Market Efforts

Sponsor Funds the LLC for:
• Registry development
• Maintenance
• Accounting and Legal Expenses
• Quarterly contributions to debra of America and DEBRA International
• First international online patient registry, EBCare.org, launched on Feb 29, 2012 (Rare Disease Day) in collaboration with debra of America and DEBRA International
• Set up and maintenance funded by New Co.
• Registry built and maintained by Innolyst, Inc./Patient Crossroads
• Data owned by EB Care, LLC
• Patient Crossroads to provide de-identified data to New Co. and researchers approved by EBCare, LLC
• debra to actively promote registry and support patients with any questions
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Courageous Patients. Bold Effort.
Golden Age of Rare Diseases

- New companies are being formed
- Venture capital is heavily invested in rare diseases
- Animal models now available
- Gene therapy is emerging as a long term treatment
- Competing therapies are now entering the market
- Many companies now have patient advocates
Have a Game Plan

- What do I need to talk to a company?
- Whom should I talk to?
- What should I offer?
What Do I Need?

- Background on the disease
- Number of known patients
- Patient groups-formal or social media
- Key opinion leaders
- Treatment centers
- Current treatments
Helpful to Have

- Animal models
- Patient registry
- Financial resources
Contacting a Company

- Researcher with a potential treatment
- Researcher with in-depth disease knowledge
- Patient/parent representing a patient community
- Six degrees of separation contact
- Business development officer
- Corporate patient advocate
- Other patient groups working with the company
- Representatives often attend key medical meetings
What Should I Offer?

- Personal experience with the disease
- Contacts to others
- Treatments currently being used
- Registry data
- Willingness to work with industry
Registry

- Increasingly used for natural history of the disease
- May serve as comparator for patients in clinical trial
- Important for companies in building financial models
- Need not be costly to develop
Coordination of Rare Diseases at Sanford

- Global Rare Disease Patient Registry for ALL rare diseases
- Collects & collates contact & clinical information
- Provides a mechanism for retrieval and dissemination
- Accelerate research into rare diseases

What makes CoRDS unique?

- No cost registry platform option for patient advocacy groups
- Opportunity for patients with no patient group
- Flexible to accommodate needs of stakeholder
- No cost to organization nor participant to enroll
- Umbrella IRB-approval, secure data collection & management
Preparing Yourself and Your Organization

- Professional Patient Advocates in the Life Sciences
- First conference May 11-13 in Sioux Falls, SD
- Two day conference featuring nationally known leaders in Rare Diseases
- Tracks for patient organization leaders and for professionals working in industry
- Prepare patient leaders to lead your organization to and through the clinical trial process
- Prepare industry professionals to assume leadership roles in their companies
Final Thoughts

- Being united is critical for success
- Infighting in organizations delays progress
- Keep your issues in the “family”
- View industry as a partner not as the “golden goose”