Partnering with Industry in Pediatric Epilepsy Research

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Disclosures

- Contracted research
  - Zogenix, Stoke, Marinus,Biopharm, Encoded

- Consulting
  - Encoded, Greenwich, Xenon, Epygenix, Invitae, Knopp, Epilepsy Study Consortium, Longboard, Asceneuron, Praxis, Supernus

- Stock options
  - Epygenix

- This is still all VERY NEW to me and what I am going to present is just my personal experience!

Outline

- Why get involved?
- How to get involved?
- How to make your involvement successful!

Why?

Exciting time for Pediatric Epilepsy!

- Many commercial sponsors are now interested in rare pediatric epilepsy research
- Pathway to FDA approval- advantages of orphan disease designation
- The sponsors generally don’t have much pediatric specific epilepsy experience
  - They need US to help with all aspects of clinical trial development
  - Recruitment in rare pediatric epilepsies continues to be a challenge

Our Role

- Help identify target populations with unmet need
- Provide realistic, real-world observations about feasibility of the target study population
  - Seizure burden
  - Seizure types
  - Inclusion of other patient centered outcomes
- If WE don’t do this, lesser qualified people will

How?

Advisory Board invitations

- Some sponsors who are new to the space may reach out to you based on prior clinical trials experience, publications, presentations at meetings
- If they reach out and this is an area of interest, consider formalizing a consulting contract.
  - Don’t give too much of your expertise away for free!
  - Institution specific rules/guidelines regarding outside consulting
- Once a consultant, do not be afraid to give your honest opinion

Feasibility Questionnaires

- Don’t delete emails prematurely with this in subject line
- If not involved in initial protocol discussion, may get invited for possible participation as a site
- Often required to sign a CDA prior to being given full protocol for review
    - Again institutional specific rules as to who can sign off on this but if it has the potential for contracted research, likely institutional sign-off required
- Complete the questionnaires and if you don’t know the answer to some questions, ie pharmacy, core lab-reach out to colleagues who do know
- Be honest about patient cohort and recruitment goals

You have been selected… now what!?

Don’t do this for “protected time”

- Industry sponsored clinical trials research take a lot of time
- A lot of that time spent is not very interesting
  - Must believe in the research being done
  - Have a connection to the patient population
- Time spent will often be more than what is paid for by sponsor (more later)

The Value of a Clinical Research Coordinator

- The success of your site will live or die based on the quality of your CRC
- Often difficult to get started as most projects may only require 20-30% CRC effort
- Discuss with your Division chief if there are existing CRC's who need more effort OR who may have some extra bandwidth to help
- Should have some experience with:
  - Regulatory submissions-getting easier with more widespread use of central IRBs
  - Budget review
  - Electronic data capture systems
  - Monitoring visits

How to be a successful PI

- Advocate for yourself up front
  - Ask to be overall coordinating PI if you feel that is appropriate
  - Ask for set amount of % time for “PI oversight” in addition to per patient costs in budget
- PATIENT RECRUITMENT
  - Be proactive with your clinical group in identifying patients that meet inclusion criteria
  - Checking in a few times per month about X type of patient
  - Keep your own patient list of potential subjects even during study start-up
- Attend investigator meetings and ask questions or offer answers based on your experience
- Advocate for yourself regarding authorship on posters, publications

Budget

- Don’t be shy about asking for more than the what is included in sponsor template
  - Geographic variations in costs are huge
  - Look closely at time based effort for yourself and your CRC
  - Include line items for CRC and PI time during monitoring visits

Study oversight

- Stay involved and don’t expect your CRC to do all the work
- Regular meetings with CRC to ensure scheduling of visits are in window
- Detailed documentation in source documents
- Timely signing off on lab reports, studies-audit risk
- Even if they are not YOUR patients, they are now so take ownership of them
- Meet with study monitors even if it is just for a few minutes and ask for feedback regarding your site performance

If you build it they will come

- Continues to be an exciting time for our field
- Once you get the reputation as a good PI and site, more opportunities will very likely follow
- Don’t be afraid to say “no” if you don’t have the interest or the potential patients
- Very rewarding to be part of something from beginning to attend and contributing to a new therapy being brought to the clinics to help our patients.