The Role of Patient Organizations

Ron Bartek
President, Co-Founder
Getting to the Middle of the Valley

• Patient organizations pushing toward the middle:
  – Funding, maturing the basic, discovery science & matchmaking (pharmas almost twice as successful developing discoveries from outside sources vs. inside)
  – Developing translational tools (bio samples, tissue & organ bank, cell lines & models, animal models, high- and low-throughput screening assays, biomarkers)
  – Developing (unsexy) clinical infrastructure (natural history studies, clinical outcome measures, patient registry & communications, clinical network).
  – Clinical trial design insights from natural history and clinical experience (e.g., age range, age of onset, natural progression, therapeutic windows, tissue mosaicism, co-morbidities, medications, genetic variants, compliance)
  – Working closely with the NIH to increase support for the above and with the FDA to help prepare a better resourced, more certain, flexible regulatory pathway.
Meeting in the Middle – Crossing Together

- Pharmas searching valley for low-hanging fruit. Patient orgs growing it & trying to get Pharma to pick it earlier
- Three Case Studies:
  - Start-up biotech visited; had small molecules to improve mitochondrial function; asked which FA scientists should test them & whom to meet at NIH; we made introductions to best assay and NIH; assay reported exciting results; biotech needed help for pre-clinical development; submitted joint application to NIH RAID; first non-cancer successful application; FARA awarded $3.5M research grant & invested $1.1M; joined sponsor for pre-IND; assisted in designing clinical plan; Phase I in healthies; Phase II in FA patients showed clinically significant benefit in just 28 days; pivotal trial begins any day; FARA recruited phase II in days & will do so for pivotal trial
Meeting in the Middle – Crossing Together

-- FA investigator shows in 8-patient open-label study promising results with mitochondrial agent; FARA works with NIH to secure enough drug to conduct trials; FARA advocate and investigators serve on NIH steering committee for clinical plan & joins NIH at pre-IND meeting; NIH/NINDS conducts phase I in patients and phase II; FARA recruits both in weeks. FARA assists sponsor in planning phase III; sponsor spends $1M to recruit but FARA recruits all but one of the 70 patients; phase III is conducted in the FARA clinical network.

-- Start-up bio-tech calls & asks for assay & animal model; once data are in, arranges full-day meeting with regulatory advisers, FARA staff, clinicians, basic scientists to mine natural history, clinical experience, outcome measures to design clinical trial plan; two months later, includes FARA and leading clinician in pre-IND meeting.
None Can Cross Alone

It’s together or not at all.

Patient organizations are working hard to do all they can.

Acting alone, there is little we can accomplish. Acting together, there is little we will not accomplish!