

## INTRONN'S STORY WITH SBIRs

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- I. Intronn's background: RNA biology, a technology known as SMaRT™
  - i. Intronn is the only company doing this
  - ii. Intronn is a true "blue collar" biotechnology company
    - Intronn's technology did not come from a government or academic lab
    - Invented by a young pathologist in his living room while he was looking for employment following his training
    - He did not have a lab
    - He had never published in this field before
    - He had a good idea
    - Able to obtain some funding from private sources to determine if this technology worked
      1. it did
- II. Intronn and SBIRs
  - i. Intronn applied for its first SBIR to the NIH in the area of cystic fibrosis (CF)
    - Phase I SBIR
  - ii. Review Committee at the NIH, 18-20 experts in the field
    - Not only approved the grant, but...
    - Essentially doubled the budget
    - Intronn had three employees at the time
  - iii. Intronn met the objectives of the Phase I SBIR and applied for a Phase II SBIR
    - Different Review Committee at the NIH stated
    - "...this was one of the most innovative, thoughtful and exciting applications they ever read"
    - SBIR Phase II grant was awarded: \$2.7M over two years
    - Intronn met the objectives
      1. Results were published; cover story in the journal *Nature Biotechnology*
      2. The journal also had a commentary on our results, stating Intronn's technology "...shows promise for the treatment of cystic fibrosis."
      3. Little Intronn was on its way
- III. Intronn and Venture Capital
  - i. On the basis of its successful SBIR programs, Intronn was able to acquire funding from venture capital firms
  - ii. In 2002, Intronn submitted another SBIR application for cystic fibrosis
    - The objective was to determine if this candidate treatment for cystic fibrosis should be taken into the clinic
    - The application was reviewed by the peer review process at the NIH

- Yet another Review Committee
  - The application was approved for funding in the summer of 2003
- IV. The Lights Go Out
- i. Several months into the new study, the NIH requested a breakdown of Intronn's ownership
    - The request was due to the new interpretation made by the SBA re venture capital ownership
    - Intronn had acted in good faith; never knew of the new interpretation
      1. never informed by the NIH that new rules applied
      2. at the June, 2003 SBIR Conference held at the NIH, no mention was made of this new interpretation
        - a. The NIH presentation at this meeting stressed how SBIR grants can provide scientific validation needed for VC funding
      3. Because of venture capital investments in Intronn, the SBIR grant was rescinded
        - a. The cystic fibrosis program ended, the employees terminated
- V. Recap
- i. Intronn, a very small company, had a novel idea
  - ii. This novel idea competed with other novel ideas and received funding for three different SBIR grants
  - iii. Intronn's technology was judged to be one of the best
    - Each SBIR grant moved the program along on the path to clinical trials
    - Intronn worked hard to achieve its objectives
    - The NIH invested more than \$3M in these studies
  - iv. The SBA turned out the lights on this exciting program that offered much potential
    - I cannot say that this technology would have brought a cure to cystic fibrosis
    - My frustration is that our technology showed promise and we didn't get the chance to test it
- VI. Final Thoughts
- i. According to the current eligibility requirements, a hypothetical company with 490 employees and revenues of \$50M per year is eligible for SBIR grants
  - ii. An early stage biotechnology company with 15 employees, even three employees, with venture backing is not eligible
    - Something is wrong with this picture
  - iii. In biotechnology, especially where companies are pursuing new treatments to cure disease, the path is long and uncertain, costs are exorbitant, revenues are years and years away if they come at all
  - iv. The intent of SBIR legislation was:
    - To help small companies be more competitive
    - To stimulate innovative ideas and products in small companies
  - v. SBIRs are not entitlements

- vi. SBIRs are not corporate welfare
- vii. The NIH Director, Dr. Elias Zerhouni, has stated in a letter to SBA Director Hector Barreto that the current restrictions on SBIR grants prevent the NIH from funding high quality research
- viii. Intronn is happy to compete for these SBIR funds on the basis of scientific merit as judged by peer review, not arbitrary bureaucratic decisions
  - If our SBIR applications are not funded, we have to go back and work harder, do a better job, become more competitive
  - That's the American way
- viii. Competition is the fiber of the American culture and the American economy
  - With the very best companies competing for funding, American families, especially those suffering from diseases with no effective treatments, might find some consolation and some hope
- ix. I commend Senator Kit Bond and his colleague Representative Sam Graves and all their co-sponsors for their leadership and support for introducing legislation to restore eligibility to those biotechnology companies with venture capital backing.
- x. The American public, not just a handful of biotechnology companies, have a stake in this.