

Bridging the Chasm Between Microscope and Marketplace

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Partnering
for Cures | A **FasterCures**
Meeting

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PARTNERINGFORCURES.ORG

Introduction



Bridging the Chasm Between Microscope and Marketplace

- DART is partnered with Charley's Fund, a patient association focused on DMD
- Charley's Fund, founded in 2004, has successfully sponsored numerous projects in DMD, in academia and Biotech, avg ~\$3MM/year, but has encountered numerous obstacles to their progress
- DART was founded in 2010 by Charley's Fund to provide operating capability extending the reach and impact of Charley's Fund (Deal making, Drug Devel)
- DART is a virtual, operating company composed of former senior managers from Pharma, Biotech, CRO, FDA: in 2010/2011 we expect to conclude: acquisitions, co-development deals, and validate new clinical research technology
- DART is a replicable model to address the "valley of death" in drug development, and feed the growing interest from Pharma and VC in rare diseases

Charley's Fund has been successful in sponsoring research in DMD....



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Charley's Fund

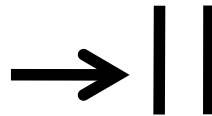
- Avg annual investment \$3MM
- Academic spin-outs, promising IP
- Sponsoring MDx mouse research to evaluate potential therapy
- Grants/sponsorship of biotechs
- "Pilot Trials Now" for existing generics with promising preclin data

But has run into increasing obstacles to further progress.....



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Charley's Fund



Obstacles

- Avg annual investment \$3MM
- Academic spin-outs, promising IP
- Sponsoring MDx mouse research to evaluate potential therapy
- Grants/sponsorship of biotechs
- "Pilot Trials Now" for existing generics with promising preclin data

- Single center investigator trials slow
- Biotechs get distracted, may underperform, shift priorities
- Promising compounds owned by "dormant" companies – no path
- Foundations restricted to "cattle prod" role – give/withhold cash



Foundation role of seed funding and "hope" less effective than ideal

DART was created to build on the success and overcome the obstacles...



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Charley's Fund



DART Therapeutics

- Avg annual investment \$3MM
- Academic spin-outs, promising IP
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- "Pilot Trials Now" for existing generics with promising preclin data

- "Virtual" Operating company staffed by former pharma, biotech, CRO, FDA
- Role: Virtual Biotech, Virtual CRO
 - Do deals, manage development
- Initial funding from Charley's Fund, long term from CRO fees, deal fees

Plans for 2010/2011 illustrate the role.. Virtual Biotech...



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Situation

DART Role

Acquisition

- Dormant biotech with clinical molecule, never tested in DMD
- Promising Preclin data
- No progress in past with Cfund

- Structure and negotiate deal
- Manage development to US IND
- Obtain financing for POC stage
- Provide critical expertise

Co-Development Deal

- Promising molecule in a portfolio
- Biotech lacks bandwidth to pursue entire portfolio
- Needs to retain future control/ buyback rights

- Structure and negotiate deal
- Provide critical expertise: Tox, FDA pediatrics
- DART contributes cash from Cfund and expert resources
- Contribute to optimal plan

Plans for 2010/2011 illustrate the role.. Virtual CRO...



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Situation

DART Role

Novel Clinical Trial technology

- Six minute walk a difficult primary endpoint
- Existing electronic muscle health assessment technology may help

- Identified promising technology
- Structure, negotiate deal
- Design, execute two stage validation studies
- Build clinical network

Clinical Trial of "Re-purposed" Generic

- Numerous generics show promise in MDx mouse model
- Unlikely to be any investor returns
- Existing clinical trial approaches very slow (single AMC)

- Provide clinical operations capability
- Use clinical network, and new trial technology, for POC studies
- Create a highly efficient clinical POC model and capability

Financing



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- Charley's Fund a 501c3 non-profit
- Initial funding for DART from Charley's Fund
 - fixed operating costs <\$200k/year; most costs program related
- DART provides mechanism for Charley's Fund investments to be managed with greater impact, more speed and control
- Charley's Fund has invested \$1MM-\$3MM in molecules in the past; now investing through DART as co-development deals, acquisitions
- Future DART funding may come from CRO fees, deal proceeds upon exit
- DART managed investments of \$1-3MM per deal done to attract follow on investments of \$10MM-\$30MM from VC, biotech

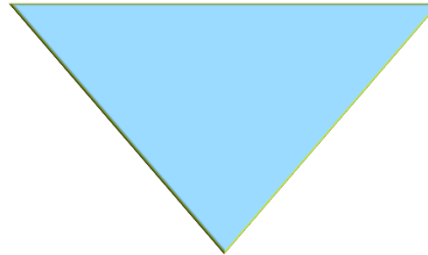
DART may be a model for adding value in many Rare Diseases....



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Patient Association/
"Discovery Research"

The "GAP"

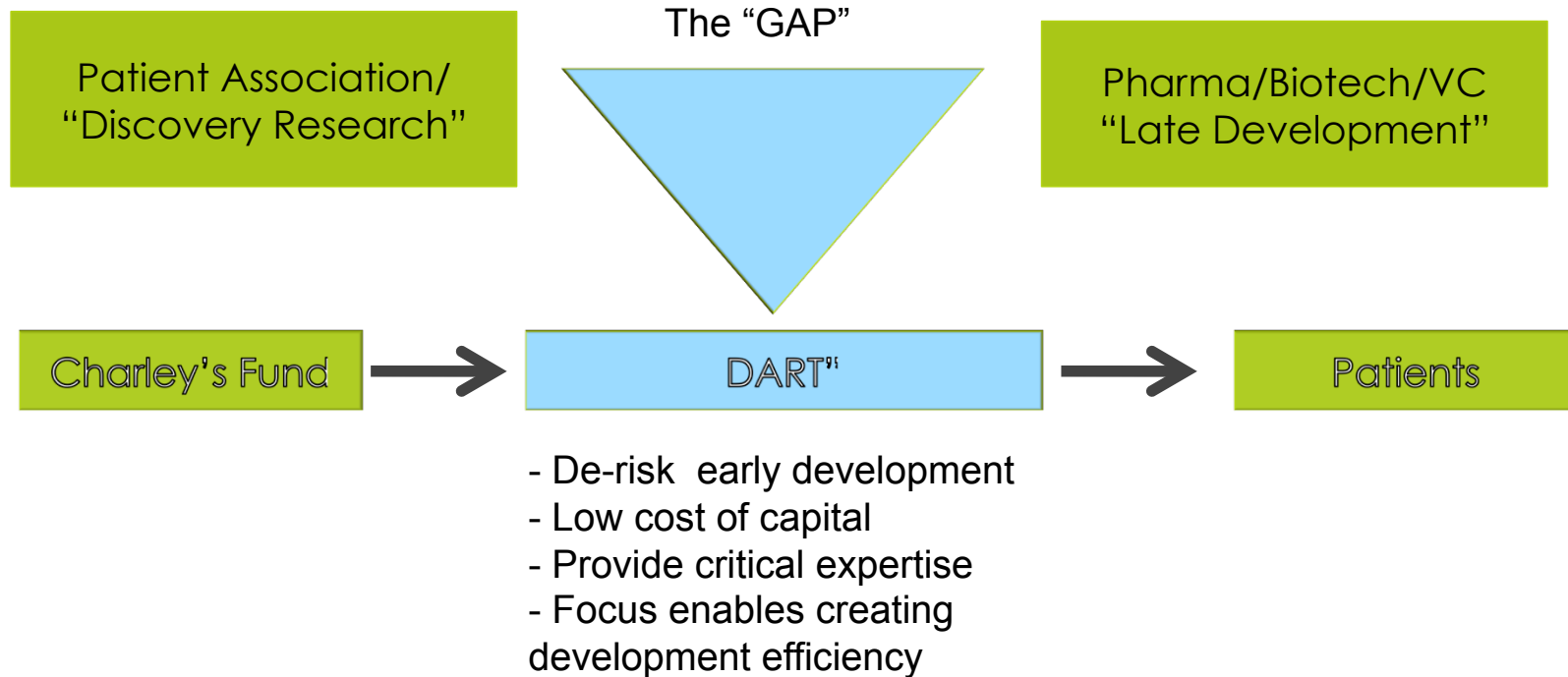


Pharma/Biotech/VC
"Late Development"

DART may be a model for adding value in many Rare Diseases....



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Summary



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- Patient Associations have made tremendous strides in the last 5-10 years; raising money, investing it with impact in Rare Diseases
 - Charley's Fund a success example in DMD
- Tremendous upsurge in interest from Pharma in Rare Diseases, and therefore also from VC investors
- DART/Charley's Fund partnership a potential model of Virtual Operating Biotech/ CRO which can bridge the gap between Patient Associations and Pharma/Biotech
 - De-risk programs at lower cost of capital
 - Provide expertise, focus to create development efficiency
 - Conduct research where prime benefit is patient – not financial
 - Multiply the impact of resources invested