ADDF ACCESS

A Novel Resource to Optimize the use of CROs by Academia and Early-stage Companies

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Alzheimer’s Drug Discovery Foundation (ADDF)
ADDF Mission:
Accelerate the Discovery of New Drugs

Basic Research
Target Validation
Pre-clinical drug discovery & Development
IND
Clinical Development
FDA/EMA Approval & Marketing

Traditional Philanthropy/Government

Biomedical Venture Philanthropy

Venture Capital/Pharma

Private Equity/Public Markets

High RISK Low

FINANCING GAP
An International Survey of Funding for Alzheimer’s Disease

Investments in Research By Category: 2008-2011

Source: International Alzheimer’s Disease Research Portfolio (IADRP)
The ADDF Translational Research Portfolio 2010-2013

- Drug Discovery Innovation Program
- Program to Accelerate Clinical Trials
- Biotechnology Development Program
ADDF Develops Innovative Resources to Accelerate Drug Discovery

The Need

• Contract research organizations (CROs) play a critical role in drug discovery and development
  ➢ Need for access to companies with assays and expertise relevant to neurodegenerative disease

• Academia and small companies have limited resources to identify, effectively evaluate and manage CRO contracts

• Successful CRO partnerships increase efficiency of programs and are required for successful partnering/securing investment
To Accelerate Translational Research in Academia and Small Biotech by Optimizing the Use of Contract Research Organizations (CROs)
The ADDF ACCESS Team

ADDF

- Howard Fillit MD, Executive Director, Chief Science Officer
- Diana Shineman PhD, Director, Scientific Affairs
- Rachel Lane PhD, Assistant Director, Scientific Affairs + ADDF ACCESS Program Manager

Advisory Board of Consultants

- Curtis Keith PhD, CSO Blavatnik Biomedical Accelerator, Harvard University
- Julie Frewson PhD, Director Scientific Alliances and Translational Programs, Biofocus
- Katya Tsaioun PhD, President, Pharma Launcher
- Marty Watterson PhD, Founder and CEO, SL Associates
- Frank Longo MD PhD, Founder, PharmatrophiX
- Steven Braithwaite PhD, Senior VP Research, Circuit therapeutics
- David Lowe PhD, Founder and CEO NeuroAssets
- Tilmann Brotz PhD, CEO Konzept Drug Development Consulting
ADDF ACCESS provides:

- **CRO marketplace**
  - Assays relevant to neurodegenerative disease

- **Resource center**
  - Strategic guidance on CRO selection and the drug discovery process

- **Access to experts**
  - Network of industry experts

- **Community**
  - Online discussion forums
ADDF ACCESS Provides a Marketplace of CROs

• CROs selected based on reputation
  ➢ Developing internal database of scientists that can provide objective references

• Due diligence to understand core capabilities
  ➢ Facilities and equipment
  ➢ Staff/technical capabilities
  ➢ Assays and experience relevant to neurodegenerative disease and CNS indications

• Negotiated discounted pricing with a number of CROs in the marketplace for ADDF referred investigators
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ADDF ACCESS Provides Educational Resources

Evaluating and selecting CROs

Appropriate due diligence of CRO capabilities, reputation, company structure, and personnel are critical in selecting the right CRO for your project. Here, we provide recommendations for what to consider during the due diligence process.

Stage 1

Set up conference calls or online meetings with 3-4 companies.

- Type of CRO – Consider what size of CRO is most appropriate for your project. Full-service, one-stop-shop large global CROs may be able to handle multiple aspects of your project, but may not be experts in every stage and may not be as well-versed in subcontracts or company services. Each of the subcontracts or CROs should be evaluated. While smaller niche CROs typically provide highly specialized techniques and flexibility, they may require that the PI manage multiple CROs to complete a project.
- Location – Consider language and cultural differences, time zone differences, the need for site visits, and material transfers.
- Company structure – Research factors such as number of years in operation, financial stability, and % employees with PhDs and industry experience. Track company mergers and acquisitions, staff turnover, and large layoffs to provide confidence in the stability of the company and the professionals working on your project.
- Experience – Determine the company’s track record with your specific disease indication and type of sponsor (academic, biotech, or pharma). Identify the core capabilities of each CRO and how regularly the techniques to be contracted are performed by technical staff.
- Validation data – Request validation data (including positive control data) for all assays and animal models to be used.
- Objective references – Talk to former clients and third-party consultants.
- Accessibility – Expect timely responses to calls and emails and enthusiasm about your project. Are they amenable to regular communication? Evaluate communication styles by requesting a confidentiality and/or master service agreements (MSA).
- Technical staff – Evaluate training of technical staff as well as study directors.
- Obtain multiple quotes – Discuss proposed budgets, payment schedule, and project milestones; are they willing to negotiate? When comparing quotes, ensure that you are comparing apples to apples. For larger studies, prepare a request for a proposal to send out to multiple CROs.
- Personal relationship – Building personal chemistry and trust with the study director responsible for the contracted studies is critical for problem solving and the success of the study. It is important that the study director feels comfortable to approach the sponsor with problems and mistakes.

Stage 2

Meet face to face with the Study Director and perform site visits (2-3 companies).

- Dig deep – Look into CRO capabilities with respect to the expertise required for specialist techniques (e.g., ICV injections, microanalysis) and ensure these techniques are performed regularly. Request references, training records, and historical control data for specific techniques to be contracted.

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ADDF ACCESS Provides a Virtual Network of Experts

**Access to experts**
- Network of drug discovery experts to provide:
  - Guidance on CRO selection
  - Guidance on experimental and program design
  - Project management
  - Opportunities for interdisciplinary collaborations

**Community (under development)**
- Discussion forum to provide:
  - Feedback on CRO performance
  - Opinions on new trends in drug discovery
  - Ask the expert
Funding Model

• Develop a consortium of funders (partner organizations) to provide support for:
  • Start-up costs
  • Program expansion
    o Annual sponsorship - tiered based on % users from each partner organization

• Partner organizations provide strategic guidance on program development
Benefits to Partner Organizations

- Access to a network of preclinical CROs working in the CNS space
- Access to discounted pricing on services
  - Example 1 ACCESS negotiated a 15% discount on chemistry services for an ADDF investigator resulting in a cost saving =$16,500
  - Example 2 ACCESS negotiated a 37% discount on experimental animals resulting in a cost saving =$36,960
- Strategic guidance to investigators on CRO selection and management and program design
Benefits to Partner Organizations (contd.)

• Integration of scientific networks

• Seat on the ADDF ACCESS Advisory Board of Consultants

• Branding on ADDF ACCESS site
## Milestones

<table>
<thead>
<tr>
<th>Year 1 (2012-2013)</th>
<th>Activity</th>
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<tbody>
<tr>
<td>ACCESS Advisory Board of Consultants formed</td>
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<td>CRO database development and due diligence (65 CROs)</td>
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<tr>
<td>Educational content: CRO selection and management</td>
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<tr>
<td>Negotiation of discounted pricing for ADDF ACCESS referred investigators</td>
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<tr>
<td>Beta site launch</td>
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Partnerships

Foundation sponsors

Other partnerships

- Harrington Discovery Institute
  - Provide drug discovery and development expertise to selected ADDF funded academic programs through the Innovation Support Center
Summary

- Seeking partnerships to provide start up funds and support expansion of ADDF ACCESS
  - Searchable database of preclinical CROs working in the CNS space
  - Strategic guidance to investigators to enable informed outsourcing decisions
  - Facilitates the formation of interdisciplinary teams

Efficient use of CROs saves time, resources and adds value to drug discovery programs