Published in final edited form as:

J Autism Dev Disord. 2015 December ; 45(12): 3900–3904. doi:10.1007/s10803-015-2636-x.

Catalysts for Change: The Role of Small Business Funders in the Creation and Dissemination of Innovation

Frederick Shic,

Child Study Center, Yale University

Daniel Smith,

Delivering Scientific Innovation for Autism LLC (DELSIA), Autism Speaks

Brian Horsburgh,

NeuroNetworks Fund

Eric Hollander,

Department of Psychiatry, Albert Einstein College of Medicine and Montefiore Medical Center

James M. Rehg, and

College of Computing, Georgia Institute of Technology

Matthew Goodwin

Department of Health Sciences, Northeastern University

Abstract

A gap exists between the expanding space of technological innovations to aid those affected by autism spectrum disorders, and the actual impact of those technologies on daily lives. This gap can be addressed through a very practical path of commercialization. However, the path from a technological innovation to a commercially viable product is fraught with challenges. These challenges can be mitigated through small business funding agencies, which are, more and more, catalyzing the dissemination of innovation by fostering social entrepreneurship through capital support and venture philanthropy. This letter describes the differences and nature of these agencies, and their importance in facilitating the translational and real-world impact of technological and scientific discoveries.

Correspondence concerning this article should be addressed to: Frederick Shic, Yale Child Study Center, Temple Medical Center, 40 Temple St Suite 7D, New Haven, CT 06510, USA; Telephone: (203) 764-5934; Fax: (203) 764-4373, frederick.shic@yale.edu. The authors have the following affiliations: Frederick Shic, Ph.D., Yale Child Study Center, School of Medicine, Yale University, New Haven, CT, USA. Daniel Smith, Ph.D., Delivering Scientific Innovation for Autism LLC (DELSIA), Autism Speaks, Boston, MA, USA. Brian Horsburgh, Ph.D., NeuroNetworks Fund, New York, NY, USA. Eric Hollander, M.D., Albert Einstein College of Medicine and Montefiore Medical Center, Bronx, NY, USA. James M. Rehg, Ph.D., School of Interactive Computing, Georgia Institute of Technology, Atlanta, GA, USA. Matthew Goodwin, Ph.D., College of Computer & Information Science and Department of Health Sciences, Northeastern University, Boston, MA, USA.

Conflicts of Interest (COIs): Frederick Shic has received research funding from F. Hoffmann-La Roche AG, and Janssen Pharmaceutica, LLC. Daniel Smith reports no conflicts of interest; DELSIA is a subsidiary of Autism Speaks, a 501(c)3 organization. Brian Horsburgh is a trustee of NeuroNetworks Fund, a 501(c)3 venture philanthropy firm, and also holds positions at Immunova LLC, Epidarex Capital, and Blue Yonder Group. Eric Hollander has received grants from Hoffmann-La Roche AG, Curemark, Brainsway, Takeda, Forest, and Coronado. James M. Rehg reports no conflicts of interest. Matthew Goodwin has COIs arising from work with Janssen Pharmaceutica, LLC, Affectiva, Inc., Empatica, Inc., and Behavior Imaging Solutions, Inc.

Keywords

technology; innovation; translational science; commercialization; business; funding; venture philanthropy

As the breadth and depth of technology studies in autism research continue to increase, it becomes timely to address how these technologies, and our knowledge of how to utilize these technologies, can be disseminated into the broader world to achieve real-world impact. More and more, autism technology researchers are turning for guidance towards mature models of innovation dissemination and adoption. For example, the pharmaceutical model of drug development usually begins with a period of preclinical drug discovery, initial studies of pharmacokinetics and safety, followed by small Go-NoGo proof-of-concept and proof-of-mechanism trials. If promising, these are followed by Phase IIb clinical trials examining efficacy. Successful Phase IIb clinical trials are then followed by at least two large-scale multicenter Phase III registration trials examining efficacy and safety, followed by post-marketing Phase-IV trials in a more real world setting. Additional studies might also examine effectiveness, impact on quality of life, and real-world dissemination. This development process is well-understood, and for promising targets can be well-funded by industry if, for example, the case can be made that the treatment addresses an unmet need and/or has advantages over existing treatments and would offer the potential to realize an adequate return on investment. In the area of bioengineering, programs such as Stanford's Biodesign, Georgia Tech's Institute for Bioengineering and Bioscience, and UCSF's Master of Translational Medicine have created effective models for public-private collaboration in the development and deployment of biotechnology solutions for health conditions.

By contrast, the model for autism technology development is more poorly understood, and often more poorly funded. Here the development path is still emerging.

In the lifecycle of a traditional translational science success story, (a) progress in basic science leads to novel treatments which are distilled via (b) clinical trials, the results of which (c) are then disseminated or made more broadly available. This process is typically well defined: research funding organizations, both public and private, offer opportunities for financial support; the ideas are reviewed and culled on the basis of scientific merit by domain experts; the trials and evaluation metrics, often clear from initial proposals, are implemented; finally, results are collected and reported. Moreover, in traditional areas of translation, research on basic science and technology already occurs in a context which incorporates an understanding of both the medical needs and challenges and the complex realities of care delivery. For innovative new technologies targeting the needs of individuals with developmental issues, there is a lack of an effective bootstrapping model for funding scientific development, and the connection between the technology community and the community of mental health researchers and clinicians is more tenuous. The source of novel technologies is often an academic research program in engineering or computer science which is focused on developing novel methods which improve performance in well-defined tasks with clear performance metrics (sometimes chosen somewhat arbitrarily). Most of the work is done by graduate students, with funding agencies such as the NSF placing a

premium on student training. In general, the resulting innovations are constrained by the need to produce novel improvements upon the current state-of-the-art in the context of a Ph.D. dissertation or Master's project.

In this context, “finishing the job”, by carrying novel developments to real-world use is frequently not a priority. For academic researchers, such an undertaking is challenging for several reasons. Researchers who are technology experts may lack the expertise and the access to the clinical community that translation requires. Funding mechanisms for medical innovation are often too risk-averse to support the exploitation of early-stage technologies, while technology-focused funding agencies and publication venues frequently lack the expertise to evaluate health-related applications with sufficient clinical rigor, and may not even value them. In these cases, the ideal reviewer needs to be able to evaluate innovation, merit, and rigor from both a technical and health outcomes perspective, and such individuals are rare, particularly in the domain of mental health. However, in appreciation of these problems, public and private funding entities have begun to provide backing to multi-disciplinary efforts across technology and health fields, bringing together a collective expertise traditionally absent in solitary disciplines. An example is the Smart and Connected Health (SCH) Program at the NSF, which is a collaboration with several NIH institutes including NCI, NICHD, and NIBIB (National Science Foundation, 2013). SCH proposals are evaluated in accordance with both NSF and NIH merit-review criteria. Such an approach is necessary to provide opportunities for advancing state-of-the-art technologies paired with rigorous clinical science. Another example is the Global Grand Challenges Program at the Bill and Melinda Gates Foundation, which solicits proposals to develop end-to-end technology-based solutions to a carefully-scoped set of global challenges in health and well-being. Example projects include sustainable vaccine manufacturing and tracking the global burden of antimicrobial resistance.

The process of transforming technologies into useful clinical tools with real world utility and impact is the most challenging and vulnerable step in the translational science success story. In general, disseminating scientific discoveries to the broader community can be accomplished through at least three, not necessarily mutually exclusive, paths: (1) scientific reporting; (2) policy recommendation; and (3) commercialization. While the first two dissemination paths are important for science and for the community, the most promising route to real-world, self-sustaining dissemination and impact for technology is through commercialization. However, there are multiple barriers in making the leap from a positive results in the laboratory or clinic to a product. These barriers include logistical challenges such as (a) difficulties in minimizing production costs and manufacturing at scale; (b) marketing, advertising, and promoting the product to increase its visibility in the market; (c) delivery of the solution to the end user through the development of appropriate sales and distribution channels; and (d) supporting the product through the lifecycle of its use by end-users who may vary substantially in their expertise, understanding, and expectations. Market challenges also exist, such as (1) being able to accurately characterize needs and market demand; (2) understanding the competition landscape and the niche of the product in the development and market ecosystem; and (3) reacting to market shifts and changes in the ecosystem due to competition or other unforeseen events. For technologies that aim to

address health issues, such as those designed for autism research, creating, enacting, and leveraging systems for measuring impact and effectiveness are both critical and non-trivial.

At this phase it is imperative to bridge a very practical gap. While research into understanding the effectiveness of the technology continues to be incredibly important, the focal needs shift away from science, and therefore leaves what was once purely the domain of scientists and researchers. Different expertise needs to be brought to bear, and funding is needed to support the transition from theory to practice. At this point, business funders become the catalysts for effecting the goal of change.

For niche markets, such as autism-focused technologies, a realistic path for creation of a sustainable business model is to begin with small businesses. Several different classes of funding entities can aid in the process of business creation around more risky technologies focused on health-related goals. These include (1) governmental organizations, such as the United States governmental Small Business Innovation Research (SBIR) and Small Business Technology Transfer (STTR) programs, which can provide capital to emerging health-oriented companies while simultaneously providing funding for pairing product development with research aimed at exploring the product's utility; (2) public or private disease-specific or patient advocacy foundation funding vehicles, such as Autism Speaks' DELSIA (Delivering Scientific Innovation for Autism LLC), which focus on providing support to emerging technologies that are pertinent to families and individuals affected by certain disorders or health issues; and (3) venture capitalists, and more and more often a new breed of venture philanthropists, who may identify and invest in innovation using capital funds in order to provide a return to partners as well as advancing the goal of improving the quality of life of affected individuals and families.

These catalytic entities will often form a symbiotic relationship with businesses, offering financial support (e.g. capital to improve infrastructure, to help move a product to market, to fund studies to provide more conclusive evidence regarding products, or to directly fund improvement or development of a product) as well as significant and important non-financial support (e.g. providing heightened awareness of the product or technology by virtue of the selection process, a structure for defining success, access to business and market experts as well as technical and domain expertise, education on business management, and networking opportunities). However, these entities both share commonalities and differ along multiple dimensions which in many ways define the scope, scale, and nature of their influences and relationships with business partners. These dimensions include (a) their ultimate funding source; (b) their expectations on return; (c) their relationship with the business; (d) the scale of their contributions; (e) their focus; and (f) their ultimate niche in the translational and product development ecosystem. Common expectations across funding entities include: (1) funding for operations come partially from the business itself and that some form of self-sustainability is achieved; (2) a return is provided to the funding entity in some concrete, capially-defined form; (3) some formal relationship exists to manage expectations and dialogue between the funder and the funded business; (4) the scale is appropriate to the financial backing; (5) there is a strong focus on developing market opportunities, growth, and performance above and beyond the academic

pursuit of knowledge; and (6) that the specific niche they occupy is contained and appropriate to the expertise of the funding agency.

For government funding for innovative research, such as governmental SBIR/STTR programs, the (a) ultimate funding source is the tax payer. (b) The expectation on reward is more general: a business that is self-sustainable and which creates value can be seen as ultimately contributing back to the economy and, consequently, to the people served by the government. Funding provided directly back to the governmental agency is commonly not required. (c) Typically, specific research and product milestones and reporting are requested as a condition of funding, and program officers managing these programs provide oversight focusing on the delivery and completion of milestones. (d) Traditionally, the contribution of the award is fixed at the time of application and cannot be exceeded, but often the funds can provide substantial support to smaller companies for pilot studies and/or early development. (e) Ultimately, the focus of these agencies is to stimulate technological innovation more broadly, to meet governmentally-highlighted research and development needs, to provide greater opportunities for development and entrepreneurship to all citizens, and to improve commercialization and ultimate use of governmentally-funded research (Small Business Administration - Office of Investment and Innovation, 2014a, 2014b). (f) These government funding agencies can provide a path for commercialization for risky scientific innovations which may not necessarily have strong market incentives, and can help to fund the critical research necessary to demonstrate the importance or utility of a product. However, there is often an emphasis on stronger scientific evidence.

For disease and advocacy foundations, such as Autism Speaks and its “venture philanthropy” affiliate DELSIA, the (a) ultimate funding sources are typically private donors comprised of constituents concerned about a common cause, often drawn from grassroots advocacy, awareness and medical research funding-raising campaigns. (b) Expectations for returns include advancing technologies that have the potential to address unmet needs important to their constituents, which would otherwise not be funded, and have clear pathways to increased accessibility of these technologies to the constituents. It is also expected that financial returns directly to the foundation would contribute to the sustainability of starting, running and financing programs to support its philanthropic mission. (c) In contrast to governmental business funding organizations, private foundations often have more flexibility and can provide short-term or follow-up engagements with the goal of facilitating the development of promising technology product concepts for the community at large. (d) Funding may be guided by an investment thesis or decided on a case-by-case basis, and may be limited to, in some cases, enabling the development of a varied and diverse portfolio of investments. The risk-reward ratios are typically determined on the ultimate basis of “return on mission”, i.e., the potential to advance the philanthropic mission, rather than purely by financial return on investment. However, when financial return is a proxy of success, e.g. when it represents the successful delivery and benefit to patients, it may still be strongly pursued. (e) The focus is usually narrowly defined within a specific disease or symptom area or product sector, but with the general goals of supporting the primary concerns and needs of the constituent group from which funding arises. (f) These foundations and agencies can provide funding for the creation and commercialization of products that can benefit the target community, even in the absence of exceptionally

strong scientific or historical evidence. Because of the focus on the community, however, longer-range scientific or exploratory goals without more obvious practical applications may have disadvantages in funding.

Venture capitalists manage pools of capital from (a) Limited Partners (LPs), typically pension funds, endowment funds, family offices and corporate entities. (b) Venture capitalists identify and invest in innovation using capital from the fund, (c) manage the investee business at board level to exit, either selling the company or floating it on an exchange via an IPO, and providing a return to the LPs. Venture capitalists have (e) financial profit as a primary goal and (d) the scale of their contributions is unlimited but also constrained by the potential of the financial return. (f) For the most promising of targets and innovations in developmental disorders, venture capitalists provide the same set of demands and possibilities as they would for any other promising underdeveloped technology.

Thus it is often the case that, in practice, pure venture capitalism often does not address issues in neurodevelopmental disorders because, while the medical need is great and the science extraordinarily promising, the development risk is very large. The result is a “Capital Gap” and insufficient funds for translating innovation to the market. Venture philanthropy and venture philanthropists (VPs), like Neuronetworks Fund and Broadview Ventures, have emerged as a class of venture capitalism to fill in this gap. For VPs, the (a) source of capital comes typically from Family Offices or high net individuals with an interest in the disease area. (b) VPs have a double bottom line: (1) an interest in making an impact on a therapeutic area and (2) a financial return which enables investment in new enterprises. (c) VPs typically invest in research and may take an equity position or more frequently a royalty position. This model can be financially successful and is best illustrated by the Cystic Fibrosis Foundation that sold its rights to a drug it had supported through development for \$3.3 billion (Risser & Gilblom, 2014). (d) Because the pool of backers is traditionally smaller, VPs can take larger risks, while providing funding levels that are less constrained by financial return due to their prioritization of potential ultimate good. (e) In addition, while often the official funding focus of VPs is narrow, because controlling members of a VP organization are fewer, there can be greater flexibility in the classes of investments that VPs can make. (f) Ultimately, VPs can take on those developments too large-in-scale for government, potentially too theoretical for private foundations, and too risky for traditional venture capitalists.

As technologies in fields of autism and other developmental disorders continue to evolve, the catalysts for bringing technologies from theory into daily use will also continue to evolve. As a field, we are seeing this evolution day-to-day, with new organizations emerging to fill gaps in the translation of basic scientific findings in autism to practical tools. While business funding agencies often have different goals and constraints, together they form a landscape where they can complement one another. Indeed, this mosaic of different values, these distinct definitions of how to define worth, catalyzes the dissemination of technologies at different levels of both scientific and practical maturity. Ultimately, the benefit is for those with needs, such as the individuals and families affected by autism. We need different definitions of worth, and we need different perspectives, from scientists, to family members, to affected individuals, to business owners, and to everyone that forms our concerned

community, in order to map out as many routes to take us from where the technology is, to where it could be.

Multiple gaps in our understanding of the translational space still exist. We need to better understand multiple definitions of success, we need to evaluate broader patterns of technology use and effectiveness, and we need to identify the challenges and barriers that prevent promising technologies from developing into practical tools. We need a more serious and valued study of the interfaces between autism research, technology development, and accessibility. Engineering and medicine must unite in this challenge, and together move forward the discussion of their field-specific ideals, concerns, and goals of translational science with multi-disciplinary teams and cross-disciplinary exposure. Together, scientists need to make bridges to business owners, realizing that such relationships are bidirectional and require education on all sides. Business owners need to be able to identify opportunities for supporting their development and growth, and need to work tirelessly both with funders as well as with the communities they hope to serve.

Within these challenges also come new opportunities. One opportunity is to begin to design those metrics and measures that best define and help us optimize outcomes for the lives of individuals and for businesses, potentially resulting in a new generation of scientist-entrepreneurs. Such approaches could add to an active and growing community for businesses, private foundations, and individuals, providing direction towards more streamlined paths to multiple definitions of success. And within these approaches lie the potential to reach further still, gaining feedback from the communities we aim to support, such that new gaps are identified, studied, and overcome.

Acknowledgments

The authors would like to thank Claire Foster for organizing, editing, management, and comments on this piece; Logan Hart and Erin Barney for editing; and Roald Oien and two anonymous experts from the intersection of business, research, and government, for their insights.

References

- National Science Foundation. Smart and Connected Health (SCH) (NSF13543). Smart and Connected Health (SCH). 2013 Feb 26. Retrieved October 9, 2015, from <http://www.nsf.gov/pubs/2013/nsf13543/nsf13543.htm>
- Risser, D.; Gilblom, K. Cystic Fibrosis Foundation Sells Drug Royalties for \$3B. Bloomberg.com. 2014 Nov 19. Retrieved October 9, 2015, from <http://www.bloomberg.com/news/articles/2014-11-19/royalty-pharma-pays-3-3-billion-for-cystic-fibrosis-royalties>
- Small Business Administration - Office of Investment and Innovation. Small Business Innovation Research (SBIR) Program: Policy Directive. 2014a Feb 24. Retrieved from https://www.sbir.gov/sites/default/files/sbir_pd_with_1-8-14_amendments_2-24-14.pdf
- Small Business Administration - Office of Investment and Innovation. Small Business Technology Transfer (STTR) Program: Policy Directive. 2014b Feb 24. Retrieved from https://www.sbir.gov/sites/default/files/sttr_pd_with_1-8-14_amendments_2-24-14.pdf