

Developing Biobanking Policy with an Oliver Twist: Addressing the Needs of Orphan and Neglected Diseases

Brian Su

Repository Citation

Brian Su, *Developing Biobanking Policy with an Oliver Twist: Addressing the Needs of Orphan and Neglected Diseases*, 66 La. L. Rev. (2006)
Available at: <https://digitalcommons.law.lsu.edu/lalrev/vol66/iss3/6>

This Comment is brought to you for free and open access by the Law Reviews and Journals at LSU Law Digital Commons. It has been accepted for inclusion in Louisiana Law Review by an authorized editor of LSU Law Digital Commons. For more information, please contact kreed25@lsu.edu.

Developing Biobanking Policy with an Oliver Twist: Addressing the Needs of Orphan and Neglected Diseases

[N]ow that he was enveloped in the old calico robes, which had grown yellow in the same service, he was badged and ticketed, and fell into his place at once . . . the orphan of a workhouse—the humble half-starved drudge—to be cuffed and buffeted through the world,—despised by all, and pitied by none.¹

TABLE OF CONTENTS

Introduction.....	772
I. Biobanking Efforts Domestically and Abroad.....	775
II. An Embarrassment of Riches: Feast and Famine in the Global Health Trade.....	779
A. “One Pill Makes You Larger and One Pill Makes You Small:” Abandoning Neglected Diseases and Developing Lifestyle Drugs to Satisfy the Whims of the Market.....	781
B. “Please Sir, I Want Some More:” The Orphan Drug Act—A Not-Quite-Perfect Solution to Address the Famine.....	785
1. The Orphan Drug Act in the United States.....	785
2. Orphan Drug Acts Internationally.....	787
III. Initiatives to Address Neglected and Orphaned Diseases.....	789
A. Foundational Initiatives.....	792
B. Initiatives to Encourage Organization of Non- Profit Groups.....	797
1. Hearth and Home: Using the Louisiana Technology Park Model to Provide Facilities and Advice to Aid Nascent Biobanking Efforts.....	798

Copyright 2006, by LOUISIANA LAW REVIEW.

1. Charles Dickens, *Oliver Twist* 4 (First Tor ed., Tom Doherty Associates, LLC 1998) (1838).

2. Boots on the Ground: Organizing and Recruiting Donors and Leaders.....	800
C. Widening the Beaten Path: Orphan Drug Act Initiatives as a Guide to Addressing Neglected Diseases.....	804
1. Show Me the Money: Domestic and International R&D Grants	804
2. International Reciprocity and the Beijing Tea Party: No Taxation without Commercialization	804
3. The Precarious Balance of Exclusivity	805
Conclusion	808

INTRODUCTION

Centuries and an ocean removed from the life of a Dickensian orphan, Megan Crowley was born in Massachusetts on December 16, 1996 to a loving family who showered her with affection. According to her mother Aileen, “From the moment we held Megan we knew she was special and that she would always be our little princess. We had no idea at the time, however, just how special or how brave Megan would become in so short a time.”²

Megan, along with her younger brother Patrick, had been born with a rare, hereditary muscular disorder, Pompe disease, which degenerates muscle tissues and interferes with the respiratory and cardiovascular systems.³ The debilitating effects of Pompe make even the most basic efforts impossible: Megan and Patrick cannot “talk, swallow, walk, breathe or eat on their own.”⁴ While the infantile-onset forms of the disease are often fatal before the first year,⁵ the two siblings have beaten the odds though they live with

2. Kristen Scarlatelli Dooley, *A Race Against Time: Finding a Cure for Pompe’s Disease*, Trinity Magazine (Spring 1999), http://www.trinitydc.edu/news_events/mags/spring99/pompe2.html (last visited Jan. 18, 2006).

3. Genzyme Corporation, *Understanding Pompe Disease 1* (2002), available at http://www.pompe.com/global/pompe_brochure.pdf [hereinafter Genzyme].

4. Barbara Laker, *In Race Against Time, Dad Works To Find Cure*, Philadelphia Daily News, March 12, 2003.

5. Genzyme, *supra* note 3, at 6.

twenty-four hour care and are closely monitored by a team of doctors.⁶

Pompe is considered an “orphan disease,” one which affects less than 200,000 individuals in the United States.⁷ While strides have been made in developing enzyme replacement treatments for Pompe, John Crowley, Megan and Patrick’s father, works tirelessly to find a cure—“Deep down, I know in five to 10 years, no kid will ever suffer from this. If all I do in life is play some part to find a cure, then that’s OK.”⁸ In fact, the first steps to finding a cure for Pompe and a host of other diseases may lie with recent developments in bioinformatics and biobanking.⁹

As the tools of science and technology have improved exponentially in recent decades, two of the leading fields, information technology and genomics, have merged to form the forefront of biomedical technology: bioinformatics. This promising new field involves “a biological information processing system—comprising computers, databases, on-line networking, and specialized software—that has given birth to a new research paradigm in which genotypic and phenotypic information is ‘mined’ to identify genes, to model protein structure, and to discover drug targets.”¹⁰ Advances in the field have “made it possible to extract exponentially more information from any given [genetic] sample and to process voluminous amounts of data.”¹¹

This field has prompted demand for the creation of biobanks, “large collections of human tissue samples . . . applying

6. Laker, *supra* note 4.

7. Genzyme, *supra* note 3, at 5. Orphan diseases are similar to neglected diseases, that is, those diseases which are neglected because the costs of developing therapies and cures cannot be justified because the market for these medicines is too impoverished for those costs to be recouped. See generally Drugs for Neglected Diseases Initiative, <http://www.dndi.org/> (last visited Jan. 18, 2006). However, “orphan” seems to speak to the number of afflicted patients while “neglected” indicates the proclivity of researchers to study the disease, thus the two terms are not always coextensive though the Orphan Drug Act encompasses both. 21 U.S.C.A. § 360ee(b)(2) (West 1999).

8. Laker, *supra* note 4.

9. See generally Matt Fleischer, *Patent Thyself*, *The American Lawyer*, June 2001, available at 6/2001 Am. Law. 84 (a non-profit patient group, PXE International, has utilized biobanking as a means of attracting research attention).

10. David E. Winickoff, *Governing Population Genomics: Law, Bioethics, and Biopolitics in Three Case Studies*, 43 *Jurimetrics J.* 187, 189 (2003).

11. Michael J. Malinowski, *Technology Transfer in BioBanking: Credits, Debits, and Population Health Futures*, 33 *J.L. Med. & Ethics* 54, 56 (2005).

'bioinformatics' to genomics research."¹² These biobanks have become a powerful tool to aid doctors and researchers in "translat[ing] this rambling string of letters that constitute the genome map into medical meaning."¹³

Because biobanks require large amounts of genetic samples as research assets, these tissues have become inherently valuable, not only to researchers but also to tissue donors from whom the samples are obtained. Donors afflicted with diseases and afflictions previously disregarded by mainstream researchers have been dealt a powerful card in the form of their own tissues. If a policy framework was established to promote altruistic biobanking practices, donors may use their own tissues as leverage to acquire a voice in pharmaceutical research and development efforts, steering projects to target orphaned and neglected diseases.

However, while humanity may possess the scientific wherewithal to pursue biobanking, a coherent policy framework to encourage responsible and beneficial biobank development is lacking. Indeed, it has been noted that, in general, biotechnology "is likely to be an area of increasing importance, one in which both public understanding and public policy lag well behind the rapid advance of technological developments."¹⁴ Thus we are presented with a unique opportunity to achieve previously unattainable health policy objectives through incentives to encourage responsible biobanking.

Incentives are necessary because market forces will likely drive commercial pharmaceuticals to focus on diseases both dire and profitable, an approach which leaves many other disease groups by the wayside.¹⁵ Thus policy, which has been developed to promote the study of orphan and neglected diseases in industrialized countries, could be expanded internationally to address similarly situated diseases that also fall outside the realm of these commercial interests.

This paper proposes a policy encouraging the development of biobanks to aid in the research of neglected ailments and orphan diseases. This policy would advance initiatives promoting the organization of disease groups to stimulate research and raise funds, as well as grant incentives similar to those offered by the

12. Winickoff, *supra* note 10, at 189.

13. Malinowski, *supra* note 11, at 56.

14. President's Council on Bioethics, Reproduction & Responsibility: The Regulation of New Biotechnologies XL (2004), available at http://www.bioethics.gov/reports/reproductionandresponsibility/_pcbe_final_reproduction_and_responsibility.pdf.

15. See discussion *infra* Part II.A.

Orphan Drug Act, a Congressional enactment designed to encourage development of treatments for "rare diseases and conditions."¹⁶

Section I provides background on biobanking efforts, including those championing the research of orphan and neglected diseases. Section II discusses why some diseases will necessarily fall outside the realm of commercial interest. Section III proposes policy solutions and incentives which promote the development of biobanks and encourage research of neglected ailments.

I. BIOBANKING EFFORTS DOMESTICALLY AND ABROAD

Biobanks provide the means to process voluminous amounts of genetic samples and corresponding medical data, weaving a complete genotypical and phenotypical snapshot of a sampled population and creating an essential resource for researchers. However, many previous biobanking efforts are no longer useful due to the failure of organizers to collect medical records and obtain adequate informed consent from donors in order to ethically broaden the field of research objectives for which donated tissues may be used.¹⁷ Coupled with trends in biomedical science, the demand for new biobanks has been greatly enhanced. The accompanying call for genetic and medical information to fill these biobanks has never been greater.¹⁸ The most widely implemented biobanks, unprecedented in the number of donor samples they collect, are primarily governmental, commercial-governmental, or commercial-academic collaborations that promise little, if any, return for the efforts of their volunteers.¹⁹

16. 21 U.S.C.A. § 360ee(a) (West 1999).

17. *E.g.*, Malinowski, *supra* note 11, at 56; Winickoff, *supra* note 10, at 193.

18. *E.g.*, Malinowski, *supra* note 11, at 56; Fleischer, *supra* note 9.

19. The UK Biobank is largely a governmental project with contributions by non-profit organizations while the Estonian Genome Project is solely a government effort. *See generally* UK Biobank, <http://www.ukbiobank.ac.uk/> (last visited Jan. 18, 2006); Estonian Genome Project, <http://www.geeni.varamu.ee/> (last visited Jan. 18, 2006). Iceland is developing a national biobank in cooperation with deCODE, a for-profit corporation. *See generally* deCODE Genetics, <http://www.decode.com/> (last visited Jan. 18, 2006). Ardais, a for-profit corporation is a key player in the National Clinical Genomics Initiative which matches it with leading medical institutions in the United States. *See generally* Ardais Corporation, <http://www.ardais.com> (last visited Jan. 18, 2006).

By contrast, disease groups previously left out of the genomics revolution have initiated grassroots efforts using biobanks as a means to leverage the value of their own genetic samples and medical records. These efforts have stimulated research that promises tangible returns for donors by isolating the genes which cause the disorder and leading to possible treatments.²⁰

England's UK Biobank is one of the world's flagship initiatives with up to half a million projected participants between the ages of forty-five and sixty-nine. It is, by far, the largest biobanking organization with plans to include about 500,000 participants.²¹ This is unsurprising given its considerable support: among its backers include British governmental entities such as UK Medical Research and the Department of Health, as well as the Wellcome Trust, a biomedical research charity organization. The UK Biobank hopes to correlate urine and blood samples with lifestyle data to achieve "a greater understanding of genetic, lifestyle and environmental factors in health and disease . . .".²²

Similarly, the wholly governmental Estonian Genome Project has cataloged 10,000 samples to date with the aim of making "it possible to carry out research both in Estonia and outside to find

20. Press Release, PXE International, U.S. Patent Office Issues First Gene Patent to Patient Advocacy Group; Co-Inventors Include Non-scientist "Mom" (Aug. 24, 2004), available at <http://www.pxe.org/patent.html> (last visited Jan. 18, 2006).

21. Press Release, UK Biobank, Statement from Chair of Board of Directors on Chief Executive Officer Transition Arrangements (Jan. 14, 2005), available at <http://www.ukbiobank.ac.uk/news/pr/14jan05.php> (last visited Jan. 18, 2006). The UK Biobank notes that it "will be the world's biggest resource for the study of the role of nature and nurture in health and disease" and "[u]p to half a million participants aged between 45 and 69 years will be involved in the project." *Id.* Comparable efforts in Estonia have cataloged 10,000 samples. Estonian Genome Project, <http://www.geenivaramu.ee/index.php?lang=eng&sub=58> (last visited Jan. 18, 2006). More than 100,000 volunteers have agreed to provide samples to deCODE's biobank in Iceland. deCODE Genetics, From Genes to Drugs, <http://www.decode.com/main/view.jsp?branch=164430> (last visited Jan. 18, 2006). Howard University's biobank, a means to help researchers target diseases that predominantly affect peoples of African descent, hopes to "gather the genetic codes, along with personal and family health histories, of about 25,000 people." Melissa Healy, *Genetic Researchers: Race Isn't a Black and White Issue*, Miami Herald, Oct. 14, 2003, available at 2003 WLNR 6187453.

22. UK Biobank, UK Biobank Briefing Note—April 2004 2, available at http://www.ukbiobank.ac.uk/docs/long_briefing_paper.pdf (last visited Jan. 18, 2006).

links between genes, environmental factors and common diseases . . . and to apply the information gained from research in making new discoveries in genomics and epidemiology, which eventually lead to increasing the efficiency of health care.”²³

While Iceland has joined England and Estonia in the biobanking field, it has done so with commercial considerations expressly in mind, having partnered with deCODE Genetics, a private, Reykjavik-based biopharmaceutical company. As of mid-2003, the Icelandic project has cataloged medical records and genetic tissue from almost 100,000 people, or almost half of the nation’s adult population. The aim of deCODE is:

to identify the genetic causes of common diseases and to apply this information to develop new drugs and diagnostic tools. Built upon an understanding of the basic biology of human disease, these products are aimed at diagnosing and counteracting the underlying biological mechanisms of disease, not just the signs and symptoms.²⁴

Teaching hospitals, including Beth Israel Deaconess Medical Center, Duke University Medical Center, the Maine Medical Center, and the University of Chicago, are also collaborating with commercial interests in cataloging donor tissue and medical data.²⁵ As part of the National Clinical Genomics Initiative, these teaching hospitals obtain tissue samples and medical information from patients.²⁶ Each hospital treats this data as its own property and licenses its use to Ardaís Corporation.²⁷ In exchange, Ardaís grants each hospital a share of its revenue.²⁸ Ardaís then uses this data to “develop systematic, large-scale procedures to comprehensively collect, process, and store research-quality clinical materials and associated information; to provide these critical resources in highly optimized formats for efficient and robust design of biomedical research studies; and to support the research and clinical programs at each participating medical institution.”²⁹

23. Estonian Genome Project, *supra* note 21.

24. deCODE Genetics, The deCODE Population Approach, <http://www.decode.com/main/view.jsp?branch=31214> (last visited Jan. 21, 2005).

25. Ardaís Corporation, Overview, http://www.ardais.com/donor_overview.asp [hereinafter Ardaís Overview] (last visited Jan. 31, 2005).

26. *Id.*

27. Winickoff, *supra* note 10, at 208.

28. *Id.*

29. Ardaís Overview, *supra* note 25.

Howard University is also developing a prominent academic biobanking venture. Howard hopes to provide researchers with an invaluable tool to help “solve the enduring medical mystery: why black Americans seem to fall ill with so many diseases—hypertension, heart disease, prostate and breast cancer, asthma, glaucoma and obesity—more frequently than do white Americans and most major ethnic groups in the United States.”³⁰ Howard hopes to include approximately 25,000 participants in its effort.³¹

Given the broad mandates of these projects, the immense number of donors involved, and their sometimes overt commercial ambitions, it is perhaps unsurprising that participation in these initiatives typically grants tissue donors little direct benefit despite the invaluable contributions they make. For example, the Estonian project expressly relinquishes any donor interest in any subsequent discoveries,³² as does the UK Biobank,³³ the Iceland-deCODE biobanking effort,³⁴ and the National Clinical Genomics Initiative.³⁵ Howard University’s project may be a notable exception as it promises to aid researchers interested in diseases which plague the racial group of its donors.

As many of these efforts divest their donors of any interest in their own tissues, they simultaneously move toward methods of

30. Healy, *supra* note 21.

31. *Id.*

32. Estonian Genome Project, Gene Donor Consent Form (2001), available at <http://www.geenivaramu.ee/index.php?lang=eng&sub=74> (last visited Jan. 18, 2006) (“I may not request a fee for providing a tissue sample The right of ownership of the tissue sample, of the description of my state of health and of other personal data and genealogy shall be transferred to the Estonian Genome Project Foundation.”).

33. UK Biobank, Your Questions Answered, <http://www.ukbiobank.ac.uk/about/faqs.php> [hereinafter UK FAQ] (last visited Jan. 18, 2006) (“As with the majority of charity and public-sector research, participants will not be paid for taking part in the project. . . . UK Biobank will be the legal owner of the database and the sample collection. Participants will not have property rights in the samples and this will be explained at the outset before they consent to participate.”).

34. deCODE Genetics, Information for Participation in a Genetic Study of [Name of Disease], <http://www.decode.com/files/filemanager/website1/file148517.pdf> [hereinafter deCODE Consent Form] (last visited Jan. 18, 2006) (“If you decide to take part in the study, you have to relinquish any claims to such financial gains . . .”).

35. Winickoff, *supra* note 10, at 216 (“The [Beth Israel] consent form states that ‘there will be no direct benefit’ to participants in the program, but that ‘society may benefit from research using your tissue by learning more about what causes diseases, how to prevent them, how to treat them, and how to cure them.’” (citing the Ardaï-Beth Israel consent form)).

commercially exploiting these assets. For instance, UK Biobank acknowledges potential collaboration with commercial interests.³⁶

However, as Howard University's model indicates, donor interests do not always have to be swept away by the drive to commercialize. A smaller scale biobanking effort, PXE International, organized by an orphan disease group, has leveraged the value of donor materials to acquire tangible results and treatments for those who contribute.

PXE International is a non-profit organization formed to address the needs of those afflicted with the orphan disease pseudoxanthoma elasticum ("PXE"), a rare connective tissue disorder.³⁷ Founded by the parents of children afflicted by this disease, PXE International has acquired over a thousand samples and has achieved some level of success by granting access only to researchers studying and developing therapies for PXE.³⁸ Thus, PXE International has harnessed the value of its members' own biological material and used it to directly benefit those donors. This result is remarkable not only because it is able to meaningfully reward donor contributions, a result its fellow ventures have been unable to achieve, but most importantly because this reward empowers an otherwise disenfranchised disease group. The PXE International approach as a model for biobanking by patient groups is discussed more thoroughly in Part III.

II. AN EMBARRASSMENT OF RICHES: FEAST AND FAMINE IN THE GLOBAL HEALTH TRADE

The health revolution of the last 30 years, which has produced substantial gains in life expectancy and unparalleled medical advances, has left most of the world's population behind in important ways For these

36. UK FAQ, *supra* note 33 ("Will pharmaceutical companies be able to access the information? Yes. It is important that pharmaceutical companies can access the information in order to research potential new drugs and treatments.").

37. Genetics Home Reference, Pseudoxanthoma Elasticum, <http://ghr.nlm.nih.gov/condition=pseudoxanthomaelasticum> (last visited Nov. 2005).

38. Winickoff, *supra* note 10, at 224 ("PXE International has been extremely successful at attracting collaborating research groups, and there are now 17 laboratories in the PXE research consortium.").

*people, the imbalance between their needs and the availability of medicines is fatal.*³⁹

Biobanks are a means to help researchers address innumerable diseases. However, commercial biobanks will, by necessity of both market opportunities and restraints, focus their studies on diseases afflicting relatively large, affluent populations to realize a profit or at least recoup research expenditures.⁴⁰ Without a clear public policy mandate to address neglected diseases, it is likely public institutions will follow the lead set by commercial efforts, blunting another avenue for addressing neglected diseases.⁴¹ Similarly, though some orphan diseases in the United States have attracted a meaningful measure of commercial attention with the help of legislation, many other diseases remain unaddressed. A coherent public policy mandate must encourage the development of biobanking resources to aid the study of neglected and orphan diseases, particularly abroad. Not only would such a policy encourage the practice of biobanking itself among populations left out of the genomics revolution, but the very existence of these biobanks could act as an incentive to stimulate further research of treatments and perhaps even cures for these diseases.

39. Médecins Sans Frontières, *Fatal Imbalance: The Crisis in Research and Development for Drugs for Neglected Diseases* 8 (2001) [hereinafter *Fatal Imbalance*].

40. Ken Silverstein, *Millions for Viagra, Pennies for Diseases of the Poor*, *The Nation*, (July 19, 1999) available at <http://www.thenation.com/doc.mhtml?i=19990719&s=silverstein> (“‘A corporation with stockholders can’t stoke up a laboratory that will focus on Third World diseases, because it will go broke,’ says Roy Vagelos, the former head of Merck. ‘That’s a social problem, and industry shouldn’t be expected to solve it.’”) (“As Neil Sweig, an industry analyst at Southeast Research Partners, puts it wearily, ‘It’s not worth the effort or the while of the large pharmaceutical companies to get involved in enormously expensive research to conquer the Anopheles mosquito.’”).

41. *Fatal Imbalance*, *supra* note 39, at 20 (“[P]ublic sector research has increasingly focused on diseases that affect wealthy countries. There is increasing pressure for publicly funded research to have commercial applications, further reinforcing the focus on lucrative diseases [P]ublic sector policies increasingly view public research as an investment that needs to create economic value.”).

A. "One Pill Makes You Larger and One Pill Makes You Small:"⁴² *Abandoning Neglected Diseases and Developing Lifestyle Drugs to Satisfy the Whims of the Market*

In the past, while commercial research has headed off many potentially fatal conditions,⁴³ these efforts have also launched a surfeit of comparatively trivial "lifestyle" treatments targeting such ills as baldness, erectile disfunction, and wrinkles.⁴⁴ Indeed, these lifestyle drugs pursue a lucrative market; Viagra accounted for more than \$1 billion in sales for Pfizer in 2003⁴⁵ while Botox, a treatment for facial wrinkles, has garnered its manufacturer, Allergan, more than \$325 million in sales in the first half of 2004 alone.⁴⁶ As a point of reference, drugs with annual sales of more than \$1 billion are considered "blockbusters."⁴⁷

Commercial ventures can hardly be blamed for seeking out these lucrative lifestyle markets. With rising research and development costs⁴⁸ these few blockbusters not only sustain

42. Jefferson Airplane, *White Rabbit (Go Ask Alice)*, on *Surrealistic Pillow* (RCA 2003).

43. Pfizer's Glucotrol treats diabetes, Inspra treats high blood pressure and congestive heart failure, and Neurontin treats epilepsy. Merck's Zocor addresses high cholesterol and Cozaar treats hypertension.

44. Baldness treatments include Pfizer's Rogaine and Merck's Propecia. Erectile disfunction treatments include Pfizer's Viagra, Lilly ICOS' Cialis, and Bayer and GlaxoSmithKline's Levitra. Wrinkle treatments include Allergan's Botox, Q-Med's Restylane, and Inamed's Hylaform.

45. Pfizer Inc., Annual Report Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934 for the Fiscal Year Ended December 31, 2003 15 (2004), available at http://www.pfizer.com/pfizer/download/investors/financial/10k_0310_04.pdf.

46. Business Wire, Allergan Reports First Quarter Operating Results (April 30, 2004), available at <http://www.shareholder.com/AGN/ReleaseDetail.cfm?ReleaseID=134256> (reporting \$150.7 million in Botox sales for the first quarter of 2004). Business Wire, Allergan Reports Second Quarter Operating Results (July 28, 2004), available at <http://www.shareholder.com/AGN/ReleaseDetail.cfm?ReleaseID=140466> (reporting \$176.9 million in Botox sales for the second quarter of 2004).

47. Fei Mei Chan, *Potential Blockbuster Drugs*, Forbes, October 8, 2003, available at http://www.forbes.com/2003/10/08/cz_fmc_1008sf.html.

48. Estimates of expenditures vary significantly as few industry sources are willing to comment on research costs. Studies have placed typical research and development costs anywhere between \$110 and \$800 million. See Fatal Imbalance, *supra* note 39, at 17 ("The group Public Citizen . . . computes the cash outlay for new drugs at \$110 million"); Drug Store News, *Turbulent Future Awaits Pharmaceuticals*, March 23, 2003, available at <http://www>.

pharmaceutical companies, but compensate for less profitable ventures as well.⁴⁹ Sales of these blockbuster lifestyle drugs also counterbalance the financial risks which remain even after a drug has entered the market. For instance, subsequent testing of FDA-approved pharmaceuticals may show that a drug has unacceptable side effects, requiring an expensive withdrawal from the market which may also involve protracted litigation.⁵⁰ Moreover, many of

findarticles.com/p/articles/mi_m3374/is_4_25/ai_99309279 "Currently, Frost & Sullivan analysts tag the average cost of bringing a new drug to market at \$800 million."). Even ostensibly reliable sources may manifest significant bias. See Silverstein, *supra* note 40 ("The drug companies defend their extraordinary profit margins, and their neglect of tropical disease, by pointing to the risks and costs of R&D [T]he costs run to an average of about \$500 million per new drug Many public health activists believe the number is wildly inflated. PhRMA gets the \$500 million figure by extrapolating from a controversial 1987 Tufts University study that factored in such variables as 'opportunity costs'—i.e., the amount of money companies forgo by not investing their R&D funds in, say, the stock market—pegged to an outrageous 9 percent rate of inflation. One of the chief researchers on the Tufts study . . . has been heavily subsidized by the industry.").

49. Merck's Emend, which lessens the side effects of chemotherapy, accounted for a relatively meager \$15 million in sales for the first half of 2004. See Merck & Co. Inc, Other Financial Disclosures First Quarter 2004 1 (2004), available at http://www.merck.com/newsroom/pdf/1q04_other_financial_disclosures.pdf (reporting Emend sales of \$6 million in the first quarter); Merck & Co. Inc, Other Financial Disclosures Second Quarter 2004 1 (2004), available at http://www.merck.com/newsroom/pdf/2q04_other_financial_disclosures.pdf (reporting Emend sales of \$9 million in the second quarter). Pfizer's Caduet, which reduces LDL cholesterol and hypertension, garnered \$30 million in sales in the first six months of 2004. Pfizer Inc., Quarterly Report Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934 for the Quarterly Period Ended June 27, 2004 25 (2004), available at http://www.pfizer.com/pfizer/download/investors/financial/10q_0806_04.pdf.

50. Merck's Vioxx, which treated arthritis pain, was withdrawn from the market after a three year clinical study demonstrated it doubled the risk of heart attack and strokes. Vioxx accounted for \$2.5 billion in worldwide sales for Merck in 2003 and the company was expected "to take a charge of \$700 million to \$750 million in the second half of this year to cover the costs of withdrawing Vioxx, including customer returns of pills sold, lost future sales and writing off the value assigned to inventory." Milt Freudenheim, *Merck and Vioxx: The Company; A Blow to Efforts to Close In on Rivals*, N.Y. Times, Oct. 1, 2004, C1, available at 2004 WLNR 4778737. These estimates do not account for the costs of defending Vioxx-related lawsuits. Consider an Illinois class action suit which includes all Vioxx users in the state, numbering around 300,000

these lifestyle drugs are byproducts of research into genuinely life-threatening conditions.⁵¹

Nevertheless, both impoverished and small disease populations are overlooked because active investment in these groups would not be commercially sound; economic forces favor targeting the afflictions of large and wealthy populations.⁵² These market constraints prevent commercial efforts from entering realms where they would do the most good, especially in third world countries where, for instance, an estimated one million people die of malaria each year.⁵³

individuals. The only named plaintiff, Constance Oswald, filed suit even though Vioxx relieved osteoarthritis pain in her knees and feet: "My experience with Vioxx was great; it really helped me." Lori Rackl, *Illinoisans Join Parade of Suits Against Merck Over Vioxx*, Chicago Sun-Times, Oct. 5, 2004, available at 2004 WLNR 11725883. The costs of withdrawing Vioxx from the market and a recent jury verdict have both taken a significant financial toll on Merck. Since Merck has stopped selling Vioxx, "its stock has fallen almost 40 percent, cutting nearly \$40 billion from the company's market value." Alex Berenson, *Second Trial For Merck On Vioxx Begins*, N.Y. Times, Sept. 15, 2005, C1, available at 2005 WLNR 14523966. In August 2005, an Angleton, Texas jury ordered the pharmaceutical company to pay a plaintiff, whose husband died after taking Vioxx for eight months, \$253 million—though Texas law will automatically reduce the recovery to \$26 million. *Id.* Moreover, litigation is far from over as "5,000 people have already sued Merck," and lawyers expect "at least 25,000 suits to be filed eventually." *Id.* Not surprisingly, the potential cost of these lawsuits is enormous as "[a]nalysts have estimated that Merck, the third-largest American drug maker, could eventually be forced to pay as much as \$50 billion to settle Vioxx lawsuits if juries continue to rule against it." *Id.*

51. Managed Care, Q & A with Alan F. Holmer, Pharmaceutical Research and Manufacturers of America (PhRMA), *Terms of Coverage for Elderly Top Priority for Drug Industry*, April 2001, available at http://www.managedcaremag.com/archives/0104/0104.qna_holmer.html ("Most current so-called 'lifestyle drugs' were discovered while testing a medicine for a life-threatening disease. For example, a medicine that stimulates hair growth in men was discovered during the testing of a drug that was intended to treat prostate disease. The vast bulk of research dollars spent by pharmaceutical companies are directed toward life-threatening conditions.").

52. Silverstein, *supra* note 40.

53. Roll Back Malaria, Malaria in Africa, http://www.rbm.who.int/cmc_upload/0/000/015/370/RBMInfosheet_3.htm (last visited Jan. 18, 2006) ("There are at least 300 million acute cases of malaria each year globally, resulting in more than a million deaths. Around 90% of these deaths occur in Africa, mostly in young children. Malaria is Africa's leading cause of under-five mortality (20%) and constitutes 10% of the continent's overall disease burden. It accounts

This market failure has been exacerbated by failures of public policy.⁵⁴ The resulting data on drugs targeting neglected diseases is startling; in the last twenty-five years, 179 new drugs were developed for cardiovascular diseases accounting for eleven percent of the global disease burden.⁵⁵ In the same period, just fifteen new drugs were developed to treat tropical diseases and tuberculosis, diseases representing twelve percent of the global disease burden.⁵⁶ Only a meager portion of the seventy billion dollars⁵⁷ spent globally each year on research and development is devoted to these neglected diseases.⁵⁸ This disparity has resulted in the charge that ninety percent of health research resources have been devoted to just ten percent of the population, the so-called “10/90 gap” in health research⁵⁹ with undue focus given to lucrative markets such as North America, Japan, and Europe.⁶⁰

for 40% of public health expenditure, 30–50% of inpatient admissions, and up to 50% of outpatient visits in areas with high malaria transmission.”).

54. Fatal Imbalance, *supra* note 39, at 10–11, 20. (“This failure does not rest exclusively on the shoulders of the private sector. Governments hold the ultimate responsibility for ensuring that peoples’ basic health needs are met. They have the responsibility to take appropriate action when market forces fail to address these needs. In the past few decades, despite clear evidence of waning private sector interest in the diseases of the poor, government action has been inadequate A needs-based approach and consolidated public funding of R&D for neglected disease drugs could have compensated for the market failure. Instead, public sector research has increasingly focused on diseases that affect wealthy countries. There is increasing pressure for publicly funded research to have commercial applications, further reinforcing the focus on lucrative diseases.”)

55. Fatal Imbalance, *supra* note 39, at 10. “Disease burden” expresses the burden imposed on a population due to death and disability. World Health Organization, Quantifying Environmental Health Impacts, *available at* http://www.who.int/quantifying_ehimpacts/en/ (last visited Jan. 18, 2006).

56. Fatal Imbalance, *supra* note 39, at 10.

57. *Id.* at 16.

58. “Little more than US\$100 million per year” accounts for public/nonprofit/foundational spending. *Id.* at 21. In a survey of eleven pharmaceutical companies, where six of the top ten were represented, most reported spending less than 1% on the neglected diseases surveyed and only one noted that it “devoted over 15% of its infectious disease R&D budget to tuberculosis and malaria.” *Id.* at 12.

59. See generally the Global Forum for Health Research: Helping Correct the 10/90 Gap, <http://www.globalforumhealth.org/> (last visited Jan. 18, 2006).

60. Fatal Imbalance, *supra* note 39, at 16.

Indeed, statements by the pharmaceutical industry indicate there is merit to this assertion.⁶¹

B. "Please Sir, I Want Some More:"⁶² The Orphan Drug Act—A Not-Quite-Perfect Solution to Address the Famine

While the situation of most neglected diseases is dire, the status of some orphan diseases is not quite as desperate. The condition of orphan disease populations in industrialized nations, particularly in the United States, is improving though far from ideal.

1. The Orphan Drug Act⁶³ in the United States

The Orphan Drug Act⁶⁴ was passed in 1983 to address "rare diseases and conditions" which affect "less than 200,000 persons" in the U.S. or "affects more than 200,000 in the [U.S.] and for which there is no reasonable expectation that the cost of developing [the drug] will be recovered from sales in the United States of such drug."⁶⁵

The committee report considering passage of the act believed that "many more drugs for rare diseases can be developed if private drug companies become more actively involved."⁶⁶ This involvement was absent because "there [was] no governmental

61. *Id.* at 18. Fred Hassan, who was Chief Executive Officer of Pharmacia Corporation at the time, noted,

[T]he United States has become the must-win market for every pharmaceutical company. In addition, there are just six or seven other critical markets, including Japan and key countries in Europe . . . This does not mean ignoring other markets. But it does mean strategically concentrating resources and top management attention on success in the key market. Again, this is very different from our industry's approach in the past, which focused on therapeutic areas across geographical regions.

62. Dickens, *supra* note 1, at 14.

63. Intuitively, allocating significant research funds towards diseases which, by definition, do not affect large populations may seem inefficient. However, these expenditures are worthwhile, not only from a humanitarian standpoint but also because research into rare diseases often leads to treatments and cures for more common ailments. Carol Rados, Orphan Products: Hope for People With Rare Diseases, FDA Consumer Magazine, (November-December 2003), available at http://www.fda.gov/fdac/features/2003/603_orphan.html (last visited Jan. 18, 2006).

64. 21 U.S.C.A. § 360aa, 360ee (West 1999).

65. *Id.* § 360ee(b)(2). See discussion *supra* note 7.

66. H.R. Rep. No. 97-840(I), at 7 (1982).

policy and therefore no governmental mechanism, to facilitate the development of . . . drugs or vaccines . . . for which the market offers no financial reward.”⁶⁷ To rectify this situation, the Orphan Drug Act sought to change “applicable federal laws to reduce the costs of developing drugs for rare diseases, and to provide financial incentives for their development”⁶⁸ which includes research grants, tax credits, and a seven year period of marketing exclusivity.⁶⁹

These incentives have been very effective: in the ten years before the Act was passed in 1983, just ten treatments had been developed to address orphan diseases.⁷⁰ By 2003, the Orphan Drug Act had encouraged development of more than 1,100 treatments for orphan diseases with 250 approved for use in the United States.⁷¹ It has been estimated that more than twelve million patients have received drugs that would otherwise not have been developed without the Orphan Drug Act.⁷² Moreover, orphaned populations often benefit from strong, grassroots organizations such as PXE International and the Genetic Alliance which promote patient advocacy in public forums.⁷³

However, the Orphan Drug Act which has spurred these changes is far from perfect. Though the Act has stimulated significant drug development for orphan diseases, granting orphan status to more than 900 ailments,⁷⁴ many orphan diseases still have

67. *Id.* at 6.

68. *Id.* at 7.

69. Food and Drug Administration, OOPD Program Overview, <http://www.fda.gov/orphan/progovw.htm> (last visited Jan. 18, 2006).

70. National Organization for Rare Diseases, Inc., Annual Report 2003 4 (2003).

71. *Id.* This surfeit of therapies induced by the relatively modest incentives granted by the Orphan Drug Act implies that the demands of research and development may not be as onerous as pharmaceutical companies suggest—viable therapies may be developed for significantly less than \$800 million. See discussion *supra* note 48.

72. Larry Stevens, *Orphan Drug Act at 20: Big Gains, Some Strains*, American Medical News (August 4, 2003), available at <http://www.ama-assn.org/amednews/2003/08/04/gvsa0804.htm>.

73. See generally PXE International, www.pxe.org; Genetic Alliance, www.geneticalliance.org.

74. John Henkle, *Orphan Drug Law Matures into Medical Mainstay*, FDA Consumer (May-June 1999), available at http://www.fda.gov/fdac/features/1999/399_orph.html (last visited Jan. 18, 2006).

no effective therapies.⁷⁵ Even those that have been developed “play a very modest role in helping sufferers of rare disorders.”⁷⁶ In one instance, a drug developed to treat ALS, Lou Gehrig’s disease, “can extend the life of ALS patients by only about three months.”⁷⁷

Also, adequate funding remains an issue. The Food and Drug Administration, in fiscal year 2004, awarded about thirteen million dollars in grants to Orphan disease research.⁷⁸ Phase I studies are eligible for \$150,000 of funding per year while Phase II and III studies may receive \$300,000 per year.⁷⁹ However, “while the amount of funding has remained level, it’s eroded each year by inflation and increased need” and in an environment where commercial pharmaceuticals are spending hundreds of millions on research and development each year, “[o]ne hundred and fifty-thousand dollars doesn’t go far when it comes to drug research.”⁸⁰

2. Orphan Drug Acts Internationally

The Orphan Drug Act has spurred similar legislation internationally, often with mixed results.⁸¹ For instance, the European Union (“EU”) enactment is similar to that of the United States’ with provisions for, among other incentives, research grants, reduction of marketing fees, and a period of market

75. Stevens, *supra* note 72 (“Although the act has spawned 231 drugs, many orphan disorders still have no effective therapies.”).

76. *Id.*

77. *Id.*

78. Food and Drug Administration, OOPD Frequently Asked Questions, <http://www.fda.gov/orphan/faq/index.htm> (last visited Jan. 18, 2006) (“The current annual budget for funding grants is approximately \$13 million.”); Thomas Morrow, *Orphan Drug Act Treatments Deserve Full Insurance Coverage*, September 2004, <http://www.managedcaremag.com/archives/0409/0409.biotech.html> (“In fiscal year 2004, the FDA awarded a total of \$13.3 million in grants.”).

79. Morrow, *supra* note 78.

80. Stevens, *supra* note 72.

81. Obesity, Fitness & Wellness Week, *Incentives Available for Developing Treatments for Inborn Errors*, available at 2004 WLNR 681337 (“The success of this legislation was a factor leading to the 1993 orphan drug law in Japan; the 1997 implementation . . . in Australia; and, in 1999 . . . in the European Union (EU),” stated Haffner. “Today, international support for rare disease research is providing stimulus and motivation to overcome the financial barriers and encourage development of treatment for very rare diseases throughout the world.”).

exclusivity of ten years, three years longer than its American equivalent.⁸²

However, the EU's orphan drug act has come under fire because "150 medicines have been formally designated as of special value by the scheme, [though] so far just 11 of these products have received a marketing authorization and are available to patients."⁸³ According to a group of European biotechnology firms, this impasse is because of:

"a lack of . . . coherence in the policies applied by the different regulatory and national authorities—during the long process from research bench . . . to the patients' bedside." Above all . . . there is a need to speed up the granting of marketing authorisations and to ensure availability and affordability of novel treatments.⁸⁴

Japan's orphan drug law was also drafted with an eye toward its American counterpart and offers similar benefits to researchers: funding grants, tax reductions, priority placement on the regulatory body's examination schedule, and ten years of marketing exclusivity.⁸⁵ However, the Japanese law differs from its American and European counterparts in pricing and accessibility.

Like its counterparts, the Japanese orphan drug enactment is not perfect. Commentators have noted that the "U.S. system already contains many buffers against potential harms due to exorbitant prices," and "while the price of U.S. orphan drugs is high [despite these buffers], the price of Japanese orphan drugs is much greater . . . often two or three times that of the U.S. price."⁸⁶ But "since the medical coverage for Japanese citizens includes prescription drugs, the higher prices are spread over the entire nation."⁸⁷ While sweeping changes to prescription drug cost structures would undoubtedly prove unwieldy and controversial, this approach to medical coverage may resolve issues of orphan

82. European Agency for the Evaluation of Medicinal Products, Orphan Medicinal Product Designation in the European Union 2, <http://www.emea.eu.int/pdfs/human/comp/leaflet/661801En.pdf> (last visited Jan. 18, 2006).

83. Europe Information Service, Biotech Firms Seek Better Conditions, http://www.ebe-efpia.org/about_ebe/inpress/pdf/250603eis.pdf (last visited Jan. 15, 2006).

84. *Id.*

85. Gina M. Cavalier, *Pushing Parentless Pharmaceuticals: Toward an International Home for "Orphan Drugs" and a Cure for "Zebra" Diseases*, 27 *Law and Pol'y Int'l Bus.* 447, 450–51 (1996).

86. *Id.* at 458.

87. *Id.*

drug affordability in both the United States and the European Union.

Even with these various orphan drug acts, the situation of orphan disease groups remains less than ideal. The status of neglected disease groups, especially in impoverished nations, is critical; the development of biobanks may be the first step to ushering in many new therapies for these illnesses.

Yet, there is little indication that the market constraints which have impeded research on orphan and neglected diseases in the past can be transcended by the mere introduction of new technology. Commercial interests will likely continue to employ new technological means for ends similar to the old.⁸⁸ Thus it seems prudent to leave commercial biobanking efforts to their own devices while asserting national and international policy to encourage small and impoverished populations to create biobanks that may attract academics and even previously uninterested commercial researchers.

III. INITIATIVES TO ADDRESS NEGLECTED AND ORPHANED DISEASES

*The enjoyment of the highest attainable standard of health is one of the fundamental rights of every human being without distinction of race, religion, political belief, economic or social condition.*⁸⁹

Developing a more charitable biobanking policy does not necessitate substantial subsidies or gratuitous donations; significant progress does not hinge on charitable giving *per se*. While conventional stimuli such as tax incentives and research grants would promote biobanking research, the biobanking concept already grants an asset to small and impoverished disease groups by rendering their medical data and genetic samples valuable to researchers.⁹⁰ Thus, while biobanking itself may not drastically

88. See Rebecca J. Morris et al., *Capturing and Profiling Adult Hair Follicle Stem Cells*, *Nature Biotechnology* 411–17 (April 22, 2004), available at <http://www.nature.com/nbt/journal/v22/n4/full/nbt950.html> (last visited Jan. 18, 2006); Jennifer Warner, *Stem Cells Can Regrow Hair*, WebMD, March 15, 2004, <http://aolsvc.health.webmd.aol.com/content/article/83/97894.htm> (last visited Jan. 18, 2006).

89. The World Health Organization (WHO) Const. pmbl. [hereinafter WHO Constitution].

90. According to Professor Malinowski, “biobanking is a means for groups traditionally excluded from biomedical R&D to inspire research on their

reduce the cost of pharmaceutical research and development,⁹¹ it nevertheless allows patient groups to cultivate a portfolio of valuable assets including medical and genetic data, and even gene patents. The groups are then able to direct the costs by negotiating with those most willing to pay: commercial and academic interests eager to gain access to their biobanks and patent assets.

To this end, policy initiatives must encourage the organization of these groups left out of the genomics revolution to maximize their biological assets and political clout to attract research interest. Cues may be taken from the technology transfer research and development experience to help organize patients afflicted with orphaned and neglected diseases to maximize biobanking assets.⁹² In cases where self-promotion fails or requires additional assistance, governmental entities could provide incentives to induce research similar to those offered by the Orphan Drug Act.

The groups most likely to benefit from these initiatives may be orphan disease groups in industrialized nations and neglected diseases groups in impoverished areas. In these instances, PXE International's efforts may pose the archetype for future biobanking efforts organized by patient groups.

Guided by patients and the relatives of patients, those with the largest stake in finding therapies and cures, PXE International recruits researchers who contract on the non-profit organization's terms. These researchers must "sign a contract giving PXE International co-ownership of any patent that ensues from study of their tissues"⁹³ and the organization's stewardship over its closely held biological portfolio has yielded significant success. In 2000, four years after PXE International was incorporated, collaborating researchers at the University of Hawaii were able to isolate the PXE gene.⁹⁴ With the assistance of pro bono counsel and a war

ailments, and to do so on their terms—meaning with thoughtful, negotiated, and sound contractual arrangements grounded in the voluminous body of technology transfer and development arrangements and agreements associated with the genomics revolution." Malinowski, *supra* note 11, at 58.

91. This may not be as astronomical as some studies have suggested. See discussion *supra* notes 48 and 71.

92. See generally Ardaís Corporation, <http://www.ardais.com>. Ardaís uses a licensing structure to cultivate its relationship with donor hospitals.

93. Fleischer, *supra* note 9.

94. *Id.* ("In February 2000, PXE International received good news from the University of Hawaii. Pathobiologist Charles Boyd, who had been working with the organization for nearly six years, stretching back to when he had been at Rutgers University in New Jersey, had isolated the crucial gene.").

chest of \$150,000 “built up by swim-a-thons and bake sales,”⁹⁵ PXE International was able to share the gene patent⁹⁶ with the university as well as negotiate favorable licensing and royalty schemes, lowering costs of potential PXE medications.⁹⁷

PXE International’s success in rallying an otherwise powerless disease group may be attributed to savvy management of a closely held biological and medical information portfolio, the ability to directly steer efforts to advance PXE research milestones, as well as sage legal advice which formed a foundation for these efforts.⁹⁸ Thus, the PXE model is most capable of endowing a formerly disenfranchised disease group with a voice in the high-powered world of pharmaceutical research and development.

When coupled with the biomedical transfer principles utilized by the Beth Israel-Ardais method, which exchanges licenses to access biobanking information for funding and research, the PXE model may be able to empower many more. However, while some orphan disease groups, like PXE International in the United States, have already taken the initiative and made steps toward developing biobanks, many others may require financial and organizational assistance before they can reach such a point.⁹⁹

95. *Id.*

96. Gene patents may include “[a] purified DNA molecule isolated from its natural environment [which] is a chemical compound and [thus] patentable” Gregory P. Lekovic, *Genetic Diagnosis and Intellectual Property Rights: A Proposal to Amend “The Physical Immunity Statute”*, 4 Yale J. Health Pol’y, L & Ethics 275, 281 (2004) (citing United States Patent and Trademark Office, Dep’t of Commerce, *Utility Examination Guidelines*, 66 FR 1092-02 (2002)). Effectively, this “grants the right to exclude others, including the right to restrict the licensing of the [gene] for non-economic purposes.” *Id.*

97. Fleischer, *supra* note 9 (“[T]he group would only seek a deal that would maximize patient access to a future PXE diagnostic test or PXE treatment—one that provides a guarantee of access, a guaranteed supply, or a low cost.”).

98. *Id.* Though now dissolved, Boston-based law firm Testa, Hurwitz & Thibeault provided *pro bono* services to PXE International on a wide variety of matters ranging from incorporation and “counsel about acquiring specimens for their new bank” to filing gene patents.

99. Winickoff notes that the main difficulties in implementing PXE International-like organizations are “practical.” “First, it would require significant initial investment. Second, without a government mandate, it is unlikely that many of these organizations would emerge. In the . . . PXE [case], the pre-existence of both strong community identity and common goals generated meaningful political representation and bargaining power.” Winickoff, *supra* note 10, at 226.

Comparatively, neglected disease groups in developing countries will experience significant difficulty establishing biobanks because their environment is often impoverished and steeped in political uncertainty. However, it is this paucity of resources which makes creating attractive research tools like biobanks so critical: biobanks may be the only way to draw significant research attention to these commercially disenfranchised disease populations.

A. Foundational Initiatives

Spurred by technology transfer programs and policy incentives which encourage involvement by groups left out of the genomics revolution, biobanks could prove an effective means to rally disease groups overlooked by mainstream commercial and academic interests.¹⁰⁰ However, fundamental questions remain: issues of biological property rights, informed consent, and privacy have been extensively discussed by commentators.¹⁰¹

Property rights are especially important and, ideally, individual donors would be granted some form of property interest in their donated tissues.¹⁰² This would validate the PXE International approach which takes an *ad hoc* contractual property right approach to biological samples and promises to grant PXE sufferers a tangible benefit in exchange for the disclosure of their medical information and donation of genetic samples. By settling the legal issue, it would also allow the PXE International approach to be applied to other orphan and neglected disease groups; without a declared biological property right, efforts built on contractual property rights, like PXE International, rest on uncertain ground.¹⁰³

100. *Id.* at 224 (“A big problem for rare-disease groups has traditionally been the failure to attract researchers to study the disease The PXE strategy uses the economic value of the tissues and organization’s control over them both to further particular research goals important to the community and to correct market failures.”).

101. See Malinowski, *supra* note 11, at 58–59; Winickoff, *supra* note 10, at 224; Donna M. Gitter, *Ownership of Human Tissue: A Proposal for Federal Recognition of Human Research Participants’ Property Rights in Their Biological Material*, 61 Wash. & Lee L. Rev. 257 (2004).

102. See generally Gitter, *supra* note 101.

103. Professor Gitter notes that “the very existence of [PXE International’s] agreement invites consideration of how a court would rule if the researchers were to bring a suit alleging that their contract with PXE International is void as against public policy on the grounds that research participants cannot possess property rights in their tissue” *Id.* at 264.

Granting donors property rights to their biological material paves the way for likeminded donors to organize and create their own biobanks. Because ultimate control remains vested in the donors, they would be the ones to decide which researchers—academic or commercial—may have access to the valuable information stored within their biobanks.¹⁰⁴ This arrangement allows donors to pool their samples and collaborate with academic and commercial researchers to target their specific diseases such as in the PXE International model.

Such initiatives to associate non-profit patient groups with researchers are similar to technology transfer incentives enacted in the 1980s and 1990s to drive the creation of academic and industry partnerships “with the express intent of creating commercial incentives to apply research financed by taxpayer dollars that [were] trapped within filing cabinets in the nation’s universities.”¹⁰⁵ While the close collaboration of these two sectors, each pursuing divergent goals, has undoubtedly experienced growing pains,¹⁰⁶ the end result has been beneficial to both academia and industry.¹⁰⁷ Similarly, collaboration between non-profit patient groups holding biobank assets, like PXE International, with academic and commercial entities would not only direct research efforts toward neglected and orphaned diseases, but potentially achieve results neither could accomplish alone.

104. Winickoff notes that with PXE International, “[i]nstead of waiting for interested researchers to contact the group, the tissue trust allows donors to create research projects that they deem to be beneficial. This, in turn increases their economic bargaining power with the research entities involved.” Winickoff, *supra* note 10, at 225.

105. Malinowski, *supra* note 11, at 55.

106. Professor Malinowski notes that inexperience meant “[e]arly agreements [between universities and industry] often were less than desirable from a contemporary technology transfer perspective.” These collaborations have also created issues of overpatenting, conflicts of interest, impediments on academic communication, and new restraints on the transfer of research materials. *Id.* at 54–55.

107. Among the panoply of benefits includes greater productivity by researchers “on scales of academic accomplishment that include academic community service, teaching and publication,” the commercial application of research, and access to “proprietary enabling technologies” including “bioinformatics capabilities and databases—essential for meaningful participation in some of the most promising fields of contemporary genetic science such as genomics . . . and proteomics.” *Id.* at 55.

But close cooperation, as seen in PXE International, stands as the exception rather than the rule of biobank implementations because the model hinges on granting donors a property interest in their own tissues, an ownership interest which has been recognized only in PXE International's own contractual provisions. For instance, contractual terms used by biobanks in the United Kingdom and Iceland divest donors of any ownership in their genetic material.¹⁰⁸ Similarly, a California state decision, cited by a subsequent Florida federal opinion,¹⁰⁹ have both declined to grant biological property rights to patients.

In *Moore v. Regents of the University of California*,¹¹⁰ physicians repeatedly withdrew tissue samples from the plaintiff over a six year period, withdrawals which were ostensibly "necessary and required for his health and well-being" when in fact the physicians were secretly conducting research on the tissue and hoping to "benefit financially and competitively."¹¹¹ The California Supreme Court articulated several concerns in opposing propertization of the plaintiff's tissues to support a claim of conversion for the wrongful use of his tissue.

Primarily, the court asserted where "physicians act with undisclosed motives that may affect their professional judgment," the plaintiff's claim was more solidly based "in the well-recognized and long-standing principles of fiduciary duty and informed consent."¹¹² Also, because conversion was a strict liability tort, "it would impose liability on all those into whose hands the cells come, whether or not the particular defendant participated in, or knew of, the inadequate disclosures that violated the patient's right to make an informed decision."¹¹³ This was yet another reason to rest the claim in fiduciary duty and informed

108. UK FAQ, *supra* note 33 ("UK Biobank will be the legal owner of the database and the sample collection. Participants will not have property rights in the samples and this will be explained at the outset before they consent to participate."); deCODE Consent Form, *supra* note 34 ("If you decide to take part in the study, you have to relinquish any claims to such financial gains . . .").

109. See *Greenberg v. Miami Children's Hospital Research Institute*, 264 F.Supp. 2d 1064 (S.D. Fla. 2003) (holding that plaintiffs did not have a property interest in body tissue and genetic material donated for research to support a claim of conversion; the donated material was used to patent a disease gene and develop a commercial diagnostic test for that disease).

110. 793 P.2d 479 (Cal. 1990).

111. *Id.* at 481.

112. *Id.* at 493.

113. *Id.* at 494.

consent because those theories “protect the patient directly, without punishing innocent parties”¹¹⁴

Fears of strict liability “opening the floodgates” of litigation may be resolved via legislative channels. In proposing Congressional enactments of a “hybrid property rights/liability model,” commentators have suggested an additional element to find conversion of body tissue: a “profit” factor which could significantly rein in the fear of frivolous litigation:

In conversion cases, researchers’ participants would be entitled to compensation only if the researchers earned a profit from commercializing their tissue Despite fears about a proliferation of conversion actions, research participants likely would file lawsuits only if they were sufficiently aggrieved, and could expect a rather small damage awards [sic] if their tissue were not unique [and thus valuable to researchers].¹¹⁵

Moreover, this refusal to propertize donor tissues may be a position where law and policy have been outdistanced by science and technology in the last fifteen years. To the *Moore* court and the *Greenberg* court which followed it, the issues *sub judice* were primarily not those of research and development, but rather medical malpractice where informed consent and fiduciary duty neatly addressed the rights of the parties. Thus, the respective panels were perhaps unwilling to expand the conversion right of action because it was not anticipated their opinions would transcend medical malpractice.

It was unanticipated that commercial, academic, governmental, and non-profit organizations, entire industries, could be founded on the premise that genetic material and medical data not only has some modicum of value, but also comprise a fundamental unit of barter to be traded for more concrete assets such as capital and research participation. Public participation is vital for these ventures to succeed and lack of compensation for donors could undermine this extensive trade, potentially creating the “[w]idespread public perception that the current system is unfair[, resulting in] not only . . . mistrust between patients and doctors and a general decrease in research participation, but could also cause an overall decline in . . . support of such research.”¹¹⁶

Moreover, when these tissues play such a central role that they are “just as indispensable in the research process as chemical

114. *Id.*

115. Gitter, *supra* note 101, at 340.

116. *Id.* at 298.

reagents and other equipment used in scientific research, research participants are no less deserving of compensation than the suppliers of these materials . . . ” thus, “considerations of equity militate in favor of a system that compensates research participants for their involvement.”¹¹⁷

A biobanking policy which addresses the needs of orphan and neglected diseases must first reverse this international and domestic precedent which fails to grant research participants a property right in their own tissues before structured incentives, drawn from orphan drug acts and technology transfer programs, may be implemented to actively encourage such efforts. Further, guidance as to international implementation and oversight in developing these pro-biobanking policies may be provided by bodies such as the World Health Organization, whose basic tenets charge that “[t]he health of all peoples is fundamental to the attainment of peace and security and is dependent upon the fullest co-operation of individuals and States”¹¹⁸ and acknowledges that “[u]nequal development in different countries in the promotion of health and control of disease, especially communicable disease, is a common danger.”¹¹⁹

Grants of biological property rights with concomitant privacy safeguards and informed consent protections form the substrate¹²⁰ on which a charitable, non-profit biobanking policy can be built.¹²¹ Failing to grant donors a bargaining chip in the form of a biological property right would make it difficult for organized neglected and orphan disease sufferers to attract researchers; past experiences have shown that in a global industry dominated by Fortune 500 pharmaceutical giants and billion dollar research

117. *Id.* at 295, 298.

118. WHO Constitution, *supra* note 89.

119. *Id.*

120. “One of the main benefits flowing from patients groups’ claims to property rights in the tissue of their members is the potential for enhanced public access to diagnostic tests and therapeutics for the treatment of disease, to the extent that these groups demand some control over the licensing of the products developed from such tissue.” However, “it is clear that [patient advocacy groups] do not provide the only or even the best means of protecting the rights of research participants. For this reason, it is essential that the United States Congress recognize the right of each individual research participant to claim a property interest in his tissue.” Gitter, *supra* note 101, at 319, 322.

121. Malinowski, *supra* note 11, at 59. The protection of donors, while important even in conventional research, is heightened in the biobanking context because of the wealth of personal information which may be extracted from biological samples.

budgets, such homegrown organizations have relatively little political influence and even less financial capital.

B. Initiatives to Encourage Organization of Non-Profit Groups

Assuming the central issue of property rights in tissue may be resolved, other policy issues must be decided before biobanking is made available to neglected and orphaned disease groups.¹²² Policy must encourage disparate disease groups to coordinate and form cohesive, disease-specific, non-profit organizations which may recruit, raise funds, and negotiate with potential researchers.¹²³

Once organizations have been established, they may raise funding and attract researchers by employing technology transfer protocols similar to those used by the Beth Israel-Ardais collaboration—that is, exchanging research expertise and funding for access to their biobank assets. As the stewards of valuable biological resources, directors of these organizations may negotiate with interested academic or commercial entities to grant licenses for access to the biobank. In exchange for these licenses allowing them to closely examine extensive, correlated medical and genetic data,¹²⁴ corporations will often provide monetary compensation as in the Ardais model or research institutions may direct their work as the patient group suggests as in the PXE International model.¹²⁵

Finally, some of these non-profit organizations may still be unable to attract significant amounts of research interests. In such cases, national and international initiatives similar to the Orphan Drug Act may be required to stimulate research interest in these diseases.

122. Malinowski, *supra* note 11.

123. *Id.*

124. Fleischer, *supra* note 9 (“Scientists by nature insist on access to a better research tool—particularly one they have spotted in a competitor’s hand”).

125. “Technology transfer policy and regulation in biobanking must be crafted in a manner that recognizes the potential for populations to become sophisticated negotiators in biobanking and imposes necessary safeguards without chilling the endeavor and related research.” Malinowski, *supra* note 11, at 59.

1. Hearth and Home: Using the Louisiana Technology Park Model to Provide Facilities and Advice to Aid Nascent Biobanking Efforts

As Professor Malinowski has suggested, domestic and international agencies could set up “pilot projects . . . to facilitate biobanking to accomplish defined objectives.”¹²⁶ Here, the defined objective would be to develop biobanks which encourage research of neglected and orphaned diseases. If these pilot projects proved successful with a few initial patient groups, that is, they effectively promote the development of biobanks while attracting research attention, they may enjoy broader implementation.

The benefits conferred to patient groups by pilot projects could be fairly elaborate and “actually help to organize participation and negotiate biobanking arrangements . . . thereby developing sound contractual property rights for participants”¹²⁷ Additionally, pilot projects may need to provide entrepreneurial advice to startup biobanks for fundraising as well as strategic negotiations with established academic and commercial interests. Practical considerations such as reliable freezer storage facilities for donated tissues¹²⁸ and medical records¹²⁹ as well as office space and reliable communications systems would also be needed.

Such an arrangement may be similar to the Louisiana Technology Park,¹³⁰ an “accelerator” program which “offers an ongoing lifeline of support to nurture the early, critical phases of young, start up technology companies”¹³¹ whose logistical demands are not dissimilar from those of patient groups seeking to develop biobank assets. In fact, the Technology Park is host to Celgene’s Louisiana Stem Cell Repository.¹³² This repository

126. *Id.* at 60.

127. *Id.*

128. Fleischer, *supra* note 9. The Terrys initially used donated freezer space at Tufts University but later moved to facilities at an Arizona laboratory to house samples from 1,000 PXE patients. *Id.*

129. *Id.* (“[M]ost of what’s already collected doesn’t impress researchers. They want individual health histories and symptom descriptions too.”).

130. See generally Louisiana Technology Park, <http://www.latechpark.com/> (last visited Jan. 18, 2006).

131. Louisiana Technology Park, Three Year Review 6 (2004) [hereinafter LTP Review].

132. Louisiana Technology Park, *LTP Members*, <http://www.latechpark.com/dynaweb/1001205/ei.cfm?M=112&SM=&SC=100000&W=C&P=N&S=1001205> (last visited Jan. 18, 2006).

stores tissues much the same way a biobank would; placental stem cells are stored on site at 280 degrees below zero.¹³³

Other services are also available to “foster, encourage and promote success, at all levels for these companies, as well as to guide and ensure their future success in every possible way,”¹³⁴ such as subsidized office space, furniture, state-of-the-art information technology, as well as access to “financing, legal, marketing, public relations and Web design services.”¹³⁵ All of these incentives are pertinent to the development of nascent patient groups; capable public relations advice and webpage authoring expertise is needed for recruitment;¹³⁶ prudent financial advice is needed to most effectively apply limited resources; and sound legal advice is particularly important.

Legal advice is critical to efforts domestic and abroad, especially in jurisdictions which do not recognize a donor’s property right in extracted tissue. In addition to the typical legal transactions of a non-profit organization such as incorporation, biobanks must also draft contractual provisions which allow third parties access to biological assets while maintaining the organization’s interests in any potential breakthroughs.¹³⁷ Should research become fruitful, subsequent negotiations with third-party researchers would require savvy legal representation to ensure that the organization’s interests are protected.¹³⁸ Regardless of whether the group is associated with an “accelerator” program, that so much of a non-profit biobanks’ assets are vested in ephemeral intellectual property rights means capable legal advice is of paramount concern. Legal counsel should be a significant factor

133. John Pope, *Program Provides Stem Cell Access*, New Orleans Times-Picayune, July 16, 2003.

134. LTP Review, *supra* note 131.

135. Louisiana Technology Park, *Where Big Ideas Get Bigger*, <http://www.latechpark.com/dynaweb/1001205/ei.cfm?M=103&SM=&SC=100000&W=C&P=N&S=1001205> (last visited Jan. 18, 2006).

136. See discussion *infra* Part II.B.2.

137. See generally Fleischer, *supra* note 9. Testa, Hurwitz & Thibeault provided critical *pro bono* services to PXE International on a wide variety of matters ranging from incorporation and “counsel about acquiring specimens for their new bank” to filing gene patents. *Id.*

138. See Fleischer, *supra* note 9. PXE International and the University of Hawaii initially had disagreements regarding the best way to share the PXE gene patent as well as licensing provisions. *Id.*

for government and international entities to consider when promoting non-profit biobanking efforts.¹³⁹

The Louisiana Technology Park's supportive environment has produced positive results. As of 2004, the Technology Park housed eleven startup companies with seventy-seven employees representing a payroll of \$3.2 million.¹⁴⁰ These firms have also raised \$4.32 million in venture capital,¹⁴¹ and it is estimated that "three companies will graduate each year."¹⁴² The success of the Technology Park, coupled with the presence of the Stem Cell Repository as a tenant, suggests that similar assistance would be a great help to patient groups looking to establish biobanks, raise funds, and negotiate with academic and commercial entities.

2. Boots on the Ground: Organizing and Recruiting Donors and Leaders

Apart from facilities, funding, and advice, policy must also encourage patients to organize, a critical step to obtaining collaboration with researchers as it empowers the disease group by creating a body of potential donors and granting that body a unified voice for research and fundraising purposes.¹⁴³ Governmental initiatives to encourage organization could be as simple as disseminating information about the organization at public facilities or as sophisticated as educating organizational leaders to maximize research and fundraising opportunities.

However, the difficulties of organizing disease groups varies considerably from region to region and from disease group to disease group. Two factors may make organization of those stricken with neglected diseases in the remote areas of Africa and Asia significantly more onerous than organizing carriers of orphan

139. Matt Fleischer, *Pitfalls of Pro Se Patenting*, *The American Lawyer*, June 2001, available at 6/2001 Am. Law. 87 ("One slip in PXE International's impressive effort . . . is the application they have given to researchers who want access to their bank's blood and tissue. The document has a passage that stipulates, 'Any patent shall be applied for jointly.' The group had not sent a copy to patent counselor Patrick Waller of Boston-based Testa, Hurwitz & Thibeault until this spring. Upon hearing the passage, he pronounced the terms a little too *pro se* for his taste. 'You can't contract to make an invention,' he observed. 'I'll need to take a look at that.'").

140. LTP Review, *supra* note 131, at 28.

141. *Id.*

142. *Id.* at 29.

143. Winickoff, *supra* note 10, at 226.

diseases in the United States: availability of telecommunications infrastructure and political stability.

Developed telecommunications infrastructure is critical not only because it allows patient groups access to conventional telephony technologies but to the Internet as well, which plays a significant role in this organizational process. As one commentator noted:

[E]lectronic space remains a crucial force for new forms of civic participation, especially in its public-access portion. Non-commercial uses still dominate the Internet . . . there has been a proliferation of non-commercial uses and users. Civil society, whether it be individuals or NGOs, is an energetic presence in electronic space. From struggles around human rights, the environment and workers' strikes around the world to genuinely trivial pursuits, the Net has emerged as a powerful medium for non-elites to communicate, support each other's struggles and create the equivalent of insider groups at scales going from the local to the global.¹⁴⁴

Powerful as it is, access to this enabling technology is far from universal as this "[e]lectronic space is going to be far more present in highly industrialized countries than in the less developed world; and far more present for middle-class households in developed countries than for poor households in those same countries."¹⁴⁵

Political stability is also an important consideration for development of these non-profit organizations. One commentator has noted that while "political turmoil releases resources that can be used to found new organizations," in the long run, "entrepreneurs require political stability to engage in 'future-oriented behavior.'"¹⁴⁶ Some organizations may also require international support which means "[t]he political stability of a country, including economic and social stability, reduces the uncertainty of potential investors, and therefore may increase the

144. Saskia Sassen, *Towards a Sociology of Information Technology*, 50 *Current Sociology* 365, at 368.

145. *Id.* at 367.

146. Douglas R. Wholey & Jack M. Brittain, *Organizational Ecology: Finding and Implications*, 11 *Acad. of Mgmt. Review* 513, at 519 (citing H.E. Aldrich, *Organizations and Environments* 166 (1979), available at <http://www.unc.edu/~healdric/Bokk/1979.html>).

level of [foreign direct investment] that flows into that country.”¹⁴⁷ Moreover, “investors clearly take into account the stability of the investment environment before they commit their funds.”¹⁴⁸

In the United States, these factors resolve favorably and tend to ease the difficulties encountered by efforts which organize orphan and neglected diseases. In fact, many orphan diseases groups have already been organized to provide support and to disseminate knowledge.¹⁴⁹ A modern telecommunications network facilitates discussion between otherwise disparate members scattered about the country.¹⁵⁰ A relatively stable political environment encourages “future-oriented behavior” including the creation of patient groups. Additionally, with a developed educational system, leaders with business acumen willing to direct these organizations may be found¹⁵¹ and those without leadership experience may acquire these skills.¹⁵²

In contrast, the challenges of organizing efforts in the areas hardest hit by neglected diseases such as malaria seem insurmountable. The world’s highest malaria mortality rates are found in African nations, such as the Democratic Republic of Congo, Angola, Niger, and Sierra Leone,¹⁵³ which have been torn by strife and poverty making them ideal breeding grounds for epidemics.¹⁵⁴ In such dire situations, it is uncertain whether any

147. David W. Loree & Stephen E. Guisinger, *Policy and Nonpolicy Determinants of U.S. Equity Foreign Direct Investment*, 26 *J. Int’l Business Studies*, No. 2, 288 (1995).

148. *Id.*

149. See The Genetic Alliance, www.geneticalliance.org, for an index of organizations representing more than 800 genetic diseases, not all of which are orphaned or neglected.

150. *Id.* The Genetic Alliance touts one of the major benefits of membership being a robust network to connect stakeholders in the genetics community and facilitate access to critical resources. *Id.*

151. Laker, *supra* note 4. Megan Crowley’s father, John, held an MBA from Harvard Business School.

152. Sharon and Patrick Terry did not have technical or business backgrounds before they founded PXE International—they were “unlikely players. When they started the PXE group, he was a construction manager; she had a master’s in religious studies and was teaching home-schooled students part-time at a science museum.” Fleischer, *supra* note 9.

153. WHO, *The World Health Report 2004 148–55* (2004), available at <http://www.emro.who.int/PDF/whr2004en.pdf>.

154. Angola has been torn apart by civil war for more than a quarter century with seventy percent of its population considered below the poverty line. Since 1997, the Democratic People’s Republic of Congo has undergone significant

coordinated attempts to fight malaria by cultivating research interest would meet with much success, especially where even the prospect of day-to-day survival is uncertain.

However, it may be possible to “seed” stable and developed countries in these regions with non-profit patient organizations.¹⁵⁵ Governments in these more stable countries could encourage the development of domestic grassroots organizations by disseminating recruitment information and by fostering leadership and business acumen within patient groups.

As these organizations mature, they may be able to establish footholds in more volatile neighboring countries once the political situation has stabilized. These footholds could be as simple as establishing branches in neighboring countries to recruit donors and potential leaders to guide these offspring efforts, or as sophisticated as lobbying governmental and business officials to advance patient-group-friendly policy initiatives and legislation encouraging the development of non-profit biobanks. Well-established biobanking efforts may also provide financial aid, in addition to governmental and international funds, to offspring efforts in neighboring countries as well as organizational, financial, and legal advice.

periods of civil war, tribal conflict, and rebel gang fighting as well, drawing in neighboring states of Burundi, Rwanda, and Uganda. Similarly, civil war in Sierra Leone has resulted in tens of thousands of deaths and over one-third of its population displaced. Of the four countries with the highest malaria mortality rates, Niger is currently the most politically stable, though it is crippled by poverty. Niger ranks as one of the poorest countries in the world and suffers from minimal government services and insufficient funds to develop its resource base. Central Intelligence Agency (“CIA”), *The World Factbook 2005* (2005), available at <http://www.cia.gov/cia/publications/factbook/>.

155. Niger’s neighbor, Nigeria, currently holds a stable, civilian government with expanding and improving telecommunications assets. Senegal—in the same west African region as Sierra Leone—has a stable, multiparty democracy with a well developed telecommunications infrastructure. Namibia—which neighbors Angola—has a stable democracy as well as a “good” telephone system. However, the central African nations surrounding the Democratic Republic of the Congo have been marked by either political volatility, inadequate infrastructure, or both. This poses a particularly untenable situation for developing and organizing any non-profit biobanking organization. *Id.*

C. Widening the Beaten Path: Orphan Drug Act Initiatives as a Guide to Addressing Neglected Diseases

Incentives similar to those employed by orphan drug legislation may also be brought to bear to encourage research of neglected diseases abroad. These incentives may take the form of research and development grants, tax breaks, and marketing exclusivity.

1. Show Me the Money: Domestic and International R&D Grants

National and international initiatives may award grants to commercial and academic researchers who work with a non-profit biobanking organization and share their research priorities. Within the United States, the National Institutes of Health and the Orphan Drug Act may be appropriate financial sources.

Internationally, regional orphan drug enactments such as those of the European Union and Japan, as well as the World Health Organization in cooperation with the World Bank and the Office of Economic Development may provide grants as well.¹⁵⁶ The Global Fund, charged with fighting tuberculosis, malaria, and AIDS may also be a significant resource.¹⁵⁷ These international contributions will be most crucial in countries so impoverished they cannot effectively address diseases independently. In these areas, international efforts may serve as a proxy to national initiatives to promote biobanking.

2. International Reciprocity and the Beijing Tea Party: No Taxation without Commercialization

Tax breaks to researchers may be administered with relative ease if both the targeted disease and the research are located in the same country. However, in international scenarios where, for instance, a French researcher assisted a biobanking organization located in Nigeria, international agreements establishing a system of reciprocity in granting tax breaks may need to be arranged. While there is no direct precedent for such an international tax

156. Malinowski, *supra* note 11, at 60.

157. See generally The Global Fund to Fight AIDS, Tuberculosis, and Malaria, <http://www.theglobalfund.org>.

break structure, a somewhat similar system has been adopted in the People's Republic of China ("China").¹⁵⁸

In assessing China's attitudes toward altruistic investments, that is, those investments which rely "on the recipient's increased well-being and [do] not seek to profit at another's expense [thus] rescu[ing] those the market economy leaves behind,"¹⁵⁹ it has been noted that:

[T]he Chinese State Administration of Taxation (SAT) has held in an administrative ruling that foreign non-profit organizations that are recognized as tax exempt in their home country may be granted tax exempt status in China solely on the basis of their home country tax exempt[ion]. At least in theory, then, China has a formal and broadly stated mutual recognition provision in its tax laws.¹⁶⁰

This structure is not directly analogous with the system of international tax breaks we consider here. For instance, the Chinese structure applies to non-profit organizations working both in-country and on foreign soil rather than for-profit industries conducting altruistic R&D in cooperation with non-profit efforts abroad. However, the bare underpinnings are present: a system of international reciprocity granting tax credit for altruistic works. Implementation of such a system may take cues from the Chinese arrangement, adding flexibility to account for for-profit corporations pursuing altruistic research rather than only non-profits and applying the structure broadly to encompass efforts in many countries.

3. *The Precarious Balance of Exclusivity*

One of the most valuable Orphan Drug Act incentives—limited market exclusivity of seven years for any drugs which are developed—has also proven the most controversial and must be implemented carefully because it may act counter to policy.¹⁶¹ For

158. See generally Darryll K. Jones, *The Neglected Role of International Altruistic Investment in the Chinese Transition Economy*, 36 *Geo. Wash. Int'l L. Rev.* 71 (2004).

159. *Id.* at 72, 73.

160. *Id.* at 112–13.

161. Market exclusivity drives up the cost of drugs to, perhaps prohibitively, high rates. See Robert A. Bohrer & John T. Prince, *A Tale of Two Proteins: The FDA's Uncertain Interpretation of the Orphan Drug Act*, 12 *Harv. J.L. & Tech.* 365, 382 (1999) ("[C]ritics decry the fact that market exclusivity leads to higher prices that prohibit access to the drug."). These windfalls are for not

diseases such as malaria, tuberculosis, and AIDS, drugs which arise from these cooperative efforts should not be monopolized simply because the disease is too rampant and the need for effective treatments too dire. However, grants of market exclusivity make it easier for commercial, if not academic, interests to justify investing in the costly research and development process.¹⁶² Indeed, the market exclusivity provision is the most contentious aspect of the, otherwise universally lauded, Orphan Drug Act; critics have charged it drives up costs for medicines and grants pharmaceuticals an opportunity to reap a windfall¹⁶³ while its proponents assert that, without exclusivity, it would be difficult if not impossible for pharmaceuticals to recoup their research investments.¹⁶⁴

This delicate balancing of interests could be resolved with gradual implementation of market exclusivity. Medicines may be marketed exclusively in areas where a disease is common, but not widespread, and where a stable local economy may offset any potentially heightened costs resulting from the grant of exclusivity. Using malaria as an example, Nigeria may be an instance where the balancing of corporate interests and humanitarian efforts

insignificant amounts. As one commentator notes, a company operating under the exclusivity provision “recovered ten times its cost of producing a growth hormone for pituitary deficiencies, earning the company \$580 million on the drug alone in its first five years of exclusive marketing.” Ester Chang, *Fitting a Square Peg Into a Round Hole? Imposing Informed Consent and Post-Trial Obligations on United States Sponsored Clinical Trials in Developing Countries*, 11 S. Cal. Interdisc. L.J. 339, 357 n.151 (2002).

162. Richard A. Merrill, *The Architecture of Government Regulation of Medical Products*, 82 Va. L. Rev. 1753, 1791 n.119 (1996) (“[T]here is general agreement that the Orphan Drug Act produced the economic incentives needed to promote development of drugs for rare diseases.”); John Henkel, Orphan Products: New Hope for People with Rare Disorders, FDA Consumer (January 1995), available at <http://www.fda.gov/fdac/special/newdrug/orphan.html> (“[E]xclusivity gives sponsors legal protection against introduction of an identical competing product for seven years. This ‘shelter’ is critical to keeping many companies interested in orphans ‘Large firms need exclusivity to convince management to invest capital . . . [a]nd small-to-medium-sized companies need it to ensure stockholders that the product won’t be infringed upon by competitors.’ . . . These firms can gain a ‘quasi-patent’ under the Orphan Drug Act’s marketing exclusivity provision. Without this protection, many of these companies probably would not pursue orphans.”).

163. Bohrer & Prince, *supra* note 161; Chang, *supra* note 161.

164. Merrill, *supra* note 162.

resolves in favor of exclusivity.¹⁶⁵ Conversely, exclusivity should be denied in areas where the disease situation is pandemic and the local economy is depressed or nonexistent, such as in Niger. Often, these are the areas which need medicines the most. As the prevalence of disease abates and the economic climate stabilizes, market exclusivity restrictions may be gradually implemented in these formerly troubled areas.

However, this gradual implementation of market exclusivity raises many critical issues: What socio-economic conditions should be examined in deciding whether or not to implement market exclusivity? Must market exclusivity be manifested as a brightline, binary proposition or may it be implemented as a gradient—complete exclusivity for more prosperous nations, less complete for less prosperous nations—which better balances countervailing interests? How may a country regulate and enforce market exclusivity in regions where national borders may be porous and difficult to police? May humanitarian considerations justify the potential for heightened prices resulting from market exclusivity in more prosperous countries, which will have already shouldered much of the burden in establishing biobanks and encouraging drug development by enacting initiatives such as tax breaks and grants? If a troubled nation fails to stabilize within the period of market exclusivity, would the more prosperous nations be forced to absorb all the potential pricing disparities? How might market exclusivity of treatments and cures, produced by international cooperative efforts, be implemented under the requirements of globalization treaties such as the General Agreement on Tariffs and Trade (GATT) and Trade-Related Aspects of Intellectual Property Rights (TRIPS)? How may commercially-oriented interests be prevented from abusing market exclusivity provisions to obtain windfalls at the expense of patient health? However these issues are resolved, it is clear that if commercial and humanitarian interests are to be protected, market exclusivity for products developed from biobanking efforts cannot be avoided.

A wide variety of policy initiatives may be implemented to encourage the development of biobanks in all its stages, from the nascent phase to implementation, and active research and development. If incentives are skillfully administered to promising, self-sustaining biobanking efforts, it is more likely that patients stricken with previously orphaned and neglected diseases

165. See sources cited *supra* notes 153–54. Nigeria is in a far more favorable politico-economic situation relative to its neighbor, Niger.

will be able to gain a valuable voice and obtain research and funding with a minimum of governmental expenditure.

CONCLUSION

It is clear that orphaned and neglected disease groups are in dire need of assistance though some patient groups may be better off than others due to self-initiated fundraising and organization. Though still years away from a cure, Megan Crowley's perseverance in fighting Pompe disease and her parents' persistent efforts to raise awareness and arouse research interests embodies much of the unfaltering determination and entrepreneurial finesse required for widespread non-profit biobanking to succeed. These organizations must succeed. For every Pompe disease which has found a voice, for every PXE which has found a champion, dozens of other disease groups and tens of millions of other patients have been left behind. These multitudes have been abandoned by commercial interests which cannot justify the stratospheric research and development costs for diseases groups too minuscule or impoverished to recoup their expenditures as well as by public institutions which, all too often, set their research goals alongside those of commercial entities.

Biobanking initiatives, which have made our very biological material a valuable asset, has the potential to equalize this disparity. By encouraging grassroots organization and by vesting in donors a valuable property right in their own biological samples, millions of disenfranchised patients around the globe may be granted a voice in fundraising, and in directing research and development efforts. Once these efforts are realized, those who suffered in silence may finally be granted some access to the towers of commerce and the halls of academia from which they have been barred.

*Brian Su**

* I am deeply grateful to Prof. Michael Malinowski for his invaluable guidance and assistance in the development of this article. Also, much thanks to my family and friends for their support.