

Vitiligo: Challenges and Opportunities for Social Entrepreneurs and Communities

Yan Valle

CEO, VR Foundations, USA

*Corresponding author: Yan Valle, CEO Vitiligo Research Foundation, 1, Penn Plaza, #6205, New York, NY 10119, USA, Tel: +1-646-327-8169, E-mail: yan@vrfoundation.org

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Opinion

Every week a new article is published on vitiligo. Every week our insight into the pathophysiology of the disease is increasing. We are getting closer to solving the puzzle of this neglected disease for which there is currently no treatment. At the Vitiligo Research Foundation we envision that the combined efforts of social entrepreneurs, patients, non-profit foundations, academia and clinical researchers will lead to new treatment solutions and improved quality of life. This positive outcome will hopefully be realized within the next decade, provided, however, that we address some challenges and opportunities in the vitiligo area.

The VR Foundation was founded in 2010 by businessman and social entrepreneur Mr. Dmitry Aksenov, who is convinced that by harnessing methods generally attributed to the commercial sector, a small group of dedicated individuals could change whole sectors of the non-profit industry. Our team members are either patients and parents frustrated by the lack of progress in vitiligo and wanting to make a difference, or clinical researchers wanting to help patients solve the mystery of their skin color. We are all driven by the need to solve the enormous social and medical problem of vitiligo, a problem that has yet to be resolved.

The early years of the Vitiligo Research Foundation were tough. We had little understanding of the disease, no standardized data sets to analyze, no biosample collections to work with and no patient registry to communicate through. Our assumptions that patients with vitiligo were waiting in line for enrollment in clinical trials or experimental treatment were dead wrong.

Fast forward to the present and things have changed - big time. We have taken a giant step towards bringing together the interests of vitiligo patients and those capable of developing appropriate treatments. We have set up a network of Vitiligo Biobanks, built the Vitiligo CloudBank bioinformatics system, started an international Master-Class on Vitiligo and Pigmentary Disorders, and launched a global awareness campaign - World Vitiligo Day.

It's been quite an adventure and lots of hard work, with many setbacks along the way. However, our story is no way unique. Social entrepreneurs have led the drive to find treatments for many diseases, such as Prosenza, a biotech company from Netherlands, Genetic Alliances in the UK and the US, or the Myelin Repair Foundation from the U.S., to name a few.

Some successful stories include Spinal Muscular Atrophy Foundation, which recently entered into agreement with Novartis on some new molecules, with its only 8-member team and \$16 million research budget, or the Jackson Gabriel Silver Foundation that

produced a potential drug for a rare genetic condition called epidermolysis bullosa with seed donation of \$400 thousand.

The business model of non-profits involved in drug discovery is quite complex as available budgets are incomparable to those of the pharmaceutical industry. They work with very low overhead, share infrastructure, outsource or crowd source parts of the process, adapt their project portfolios to variable funding schemes. Their inherent shortcomings include low financial tolerance to errors in early stages of an R&D, lack of past experience in the industry, huge gaps in available knowledge base, among many.

Still, the biggest challenge for the social entrepreneurs is not financial, but human capital. Because non-profit teams are tiny compared to those of Big Pharma, members often wear several hats. People are the most important asset in R&D; they can be a key strategic advantage - but losing key experts, innovators or decision makers can also cause an irreparable damage in long-term. Finding researchers with requisite skills who are capable of considering commercial terms as much as they are considering scientific issues is not easy, even less so with skills in neglected diseases like vitiligo.

And this is more than simply a case of social entrepreneurship for the nearly one hundred million people suffering from vitiligo worldwide, many of whom now play an important role in therapy development for this neglected disease. In the process of its evolution from a passive sponsor of pre-clinical research to a more structured drug development non-profit organization, the Vitiligo Research Foundation has extensively discussed the natural history of the disease with patients, has collected a huge quantity of self-reported clinical data, including cases of the adverse and positive side-effects of different drugs.

Even in the absence of specific treatments for vitiligo, our ability to correlate academic insights, the progression of the disease and treatment outcomes has already provided effective treatment guidelines for vitiligo clinics in Georgia, Kazakhstan, and Russia.

Vitiligo is a "gateway disease" because it is a gateway to understanding all pigmentary disorders and arguably, melanoma, as an extreme reverse case of the same autoimmune process. Approximately 70% of the population carry one of over 30 genes identified to date that are related to the disease's onset and development. The unmet need for a cure is so huge and still so unrecognized that coordinated action from the global vitiligo community and pharmaceutical industry is necessary to develop a treatment.

The pharmaceutical development of a novel medication can take up to \$11 billion and up to 15 years from the time it is discovered till it becomes available to patients. The enormous cost is attributed to the

fact that large pharmaceutical companies include the expenditure of R&D, capital costs, regulatory approval and marketing, and typically a range of several other drugs failing in development, over a 10-year period. (However, the net cost of a single drug development is probably closer to a range of \$50 to \$100 million, as demonstrated by Light and Warburton in 2011.)

Regardless of the disparity in numbers, the balance between the value of new vitiligo treatment and cost of its development has not yet been established. Big Pharma is reluctant to invest millions of dollars in vitiligo R&D without a clear understanding of the disease and the market for its treatment.

Drug re-purposing, whereby drugs are used for treatment of diseases other than those for which they were originally intended, is emerging as a promising new model of drug development for vitiligo. It can potentially save up to ten years, and requires less than 10% of the behemoth budget of Big Pharma. In our multi-center clinical trials the immunomodulator drug Neovir has shown over 75% effectiveness in stopping generalized vitiligo progression, without adverse side-effects and at low cost. Unfortunately, it is only available in Russia and a handful of other CIS countries, and its producer has no current plans for its further registration by FDA or EMEA.

Despite the VR Foundation's significant and effective efforts to increase international collaboration between vitiligo researchers and biopharma to date, there is still a great deal more that needs to be. There is no single model for effective social entrepreneurship in vitiligo or other pigmentary disorders.

Some of us have gone for a patient support group with a charitable model, like VITFriends and Vitiligo Support International in the US, or AVF in France, or VITSAF in Nigeria. Others like AVRf (American Vitiligo Research Foundation) have gone for the umbrella model representing a broader segment of vitiligo community and focusing on interaction with the government and insurance companies. Yet others, like our own Vitiligo Research Foundation, have adopted the non-profit drug development model. This diversity is what makes the vitiligo community strong, because we all share the same vision: a better quality of life for vitiligo patients.

The time has come to massively broaden public awareness, and understanding of vitiligo and its impact on the society. Lack of awareness affects everything from fund-raising to the confidence of new researchers to invest time and energy in drug development. But it is not only in the field of fund-raising that patients can make a difference. Through enhanced awareness patients can be successful in encouraging healthcare systems and insurance companies to allocate resources and provide reimbursement for vitiligo treatments, which typically can be well over \$12,000 per year in direct costs for phototherapy alone.

Social entrepreneurs and leaders of vitiligo support groups need to work closely together to address core issues of this debilitating disease, a disease that knows no geographic or social boundaries.