

OUTCOME OF AND FOLLOW-UP TO THE MEETING OF THE CEO ROUND TABLE ON VITILIGO



1. The first meeting of the Roundtable on Vitiligo was held at the International Winter Consensus Conference of the World Health Academy in Kitzbuhel, on December 14, 2012, made possible by the sponsorship of the Vitiligo Research Foundation. The Roundtable brought together more than 200 participants from 12 countries, spanning the five continents of the world. They included representatives from academia, biotech, pharma, non-governmental organizations and donor agencies. The meetings were conducted in two plenary sessions and three Task Force groups. Each Task Force group meeting was dedicated to a different agenda in vitiligo; one for community, one for researchers and one for industry.

2. This report highlights and refers to meetings of all three Roundtable on Vitiligo Task Forces but does not intend to repeat in detail the conclusions of other than CEO Roundtable Task Force.

The CEO Roundtable Task Force, one of three dedicated working groups, held a discussion about cost-effectiveness and the key questions surrounding the economics of prospective vitiligo treatments, a patient-centered commercial strategy and reviewed estimates for the global vitiligo therapeutics market. This discussion paper outlines areas where business development and management is required in vitiligo therapeutics over the term of the next 5-10 years.

3. The keynote speech of the meeting, entitled “Commercial Strategies for Vitiligo,” was delivered by the VRF CEO Yan Valle, who started with the question: “Assuming the scientific advances will create opportunities for vitiligo diagnostic and therapy development, how do we facilitate its uptake in health systems?” He presented a paradigm of using academic research, biobanking and bioinformatics in a budget-focused pursuit of new therapy development in vitiligo, the most common but also the most neglected skin disease.

4. The recently reported prevalence of vitiligo ranges from 0.56% (China) to 8.2% (India), affecting up to 65 million people worldwide. Due to the shortcomings of dermatology practice, large segments of the population who have vitiligo may remain undiagnosed or unrecognized. This is due in part to the nature of the disease, which may not manifest itself dramatically in its early stages, although the long-term damage to the body may already be occurring.

5. Analysis of the global internet traffic related to vitiligo reveals two mysterious hot spots – one in Pakistan, another one in Peru. The first one is probably attributed to manufacturers and online resellers of a large number of “anti-vitiligo” treatments. Second spot in Peru is hard to explain with existing vitiligo statistics or internet data.

6. Vitiligo technically fits the FDA’s Rare Disease definition, which may lead to considerable R&D savings, but as later argued by Andy Goren (Applied Biology, USA) and Dennis J. Wright (Clinuvel, Australia) is unlikely to be approved by the FDA.

7. With only two notable exceptions, all available treatment options currently available in the vitiligo therapeutics market are generics. They primarily focus on improving skin appearance and are capable of inducing some degree of repigmentation, though none are able to manage vitiligo adequately and are ineffective in ceasing vitiligo progression and providing stable repigmentation.

8. Valle has briefly reviewed existing vitiligo pipeline. There are seven potential treatment options in the pipeline in different phases of clinical development. From these candidates, three are melanocytes transplantation therapies and the remaining four are therapeutic treatments, all with first-in-class molecules.

9. The global vitiligo therapeutics market is estimated to grow from \$1.4 billion in 2011 to \$2.7 billion by 2019. Currently 25 insurance companies in the USA have begun a reluctant move into the vitiligo territory, which was formerly ignored completely. The potential number of vitiligo patients a treatment can serve, is no smaller than the number of patients for which a blockbuster therapy can be prescribed. The only problem lies in the geographic distribution of the clients for such a treatment.

10. Photochemistry-based therapies promise a radical change of the vitiligo therapeutics market. Recent advances as reported by Prof. Andy Goren in novel chemical compositions present an opportunity to transform traditional NB-UVB therapy and increase safety, accessibility and compliance. Repurposing of the existing drugs holds promise, as noted by Dr. Igor Korobko and commented by Goren that existing reimbursement systems discourage Big Pharma from post-market investment in expanded indication and better targeting.

11. Successful commercialization of the existing vitiligo pathogenesis knowledge into products that can deliver desired outcomes remains challenging, there is no low hanging fruit in vitiligo tree. Sizable opportunities already exist for branding some of the existing therapies in a scientifically sound and patient-centered package. Provided vitiligo diagnosis is made and the product is deemed appropriate, the market niche for vitiligo may exist in perpetuity, similar to the chronic care market.

12. The incremental value of the Dx-Rx combination in vitiligo will be received by the entrepreneur who puts together and sells the innovation, although substantial scientific challenges exist in simultaneous Dx-Rx development. Valle has noted in discussions that Aliya Kasymkhanova (Kazakhstan) and Andy Goren (USA) have made inroads into evaluation of Dx and Rx combination. Prof. Goren commented that devices face different regulatory pathway and may be seen by the FDA as the way to circumvent the drug registration.

13. Key challenges in vitiligo therapy commercialization are (i) low awareness of presence of early vitiligo signs among both the general population and dermatologists, especially those in developing nations, and (ii) cost of treatment, as 80% of those surveyed online by the VRF are ready to pay no more than \$500 per year for vitiligo treatment. Initiation of early vitiligo diagnosis and bundled therapy prescription by dermatologists is perhaps the most challenging issue for the branded product developer.

14. Physical skin damage, some household cleaning items, hair color products, leather or rubber gloves and other things that are typically not suspected as having vitiligo inducing properties, may indeed trigger the onset of the disease in predisposed individuals, as noted in discussion by Dr. John Harris (USA).

15. Valle anticipates that treatments for vitiligo will have a very different economic model from that of conventional medicines. The combined therapy for vitiligo, which potentially includes novel diagnostic tools, targeted treatments and medical devices, will require a complex manufacturing, a scalable global supply and distribution chains. Early market entrants might benefit from the marketing of diagnostic tools that predict treatment response to narrow-band UVB, before the therapeutic solution is found.

16. Some entrepreneurs, most notably in Pakistan and Poland, have already started selling “anti-vitiligo” packages made by competing manufacturers. They include drugs, over-the-counter products, vitamins, food supplements and medical devices, all wrapped in a questionable treatment protocol.

17. VRF’s research indicates that in a single payer system that exists in vitiligo and arguably many other neglected diseases, incorporating the patient perspective is a continuous challenge on a pragmatic level. In non-life threatening vitiligo disease payers expect compelling evidence, which is a moving target of its own due to the different skin photo types and satisfaction levels in different manifestations of the disease.

18. Yan Valle has suggested that developing patient-centered value starts for commercial strategist with leveraging the relationships between awareness, diagnosis and treatment outcomes. Collective non-branded awareness campaign like World Vitiligo Day is a worthy commercial goal, which sets the stage and will result in a considerable lifetime value due to the potentially chronic nature of the vitiligo. Motivation branded campaign among at-risk population for diagnostic visit would increase the probability of proper diagnosis and increase chances for successful treatment with prescription of the commercially available products.

19. Valle also made the point that there are opportunities for public-private partnerships, which include greater academic involvement in generating fundamental knowledge in exploratory drug development, i.e. the work conducted by Prof. Lin Chunying (China) and her colleagues from Xijing Hospital on identifying biomarker signals.

20. The presentation was concluded with a summary. Therapy development for a disease with many different clinical manifestations, unknown etiology, modest genetic data, multiple and fragmented pathogenetical hypotheses is almost a “Mission Impossible”, but branded treatments for vitiligo already represent a sizable market, with a revenue potential similar to those of blockbuster drugs. There are many of the essential academic and clinical building blocks in place required for the development of the cost-effective Dx-Rx pair. The next step will be to link many of these assets and ensure communication between their stakeholders.

21. The deliberations, both in the plenary sessions and Task Force groups, took place in an open and constructive atmosphere. All the different stakeholders demonstrated their willingness to move forward in a spirit of co-operation and with a respect for the views of others. Towards the end of the meeting, participants stated that they would like to continue to operate in an open and participatory approach of the Roundtable on Vitiligo.

22. Many expressed the view that encouraging progress had been made during the first Roundtable on Vitiligo meeting, but that much remained to be done before the vitiligo research collaboration could be considered effective. Yan Valle proposed holding a second Roundtable meeting in about twelve months’ time, in conjunction with the World Health Academy’s annual congress. It was considered very important to maintain the momentum of this initiative in the coming months.