

Abstract: Strategic Planning for Rare Disease in the Scientific Community

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Objective: To strategically plan with a scientific advisory board in a rare disease organizations that drives and accelerates to treatments, while building valuable programs that support it.

Method: Strategic planning for the rare disease community must be a collaborative effort between patient advocacy groups and their scientific advisory boards. As a young foundation, we quickly learned how important it was to seek advice from our disease-specific scientific experts.

Results: The advisory board is a critical component of an organization's planning for both short and long-term goals. Many new rare disease organizations struggle to find direction and specific goals to achieve, while moving at an accelerated pace. Due to the inherent challenges facing a rare disorder community, such as relatively few patients spread across a large geographic area and few scientists and clinicians familiar with disease biology, it is imperative that all stakeholders work together for the best strategic outcome. To raise awareness within the medical and scientific community, we decided to begin with an international scientific conference that attracted new and expert researchers into the discussion. The benefit of sharing information jumpstarted a larger interest in our rare disorder. It has been our experience that this level of cooperation will improve outcomes for everyone involved. Discussing strategies, creating solutions, and implementing programs designed by, and for, the specific population that needs them, is how we plan to move forward as a community.

Conclusion: This presentation explains the benefits of strategically planning with a vested groups of rare disease experts. It is important that the patient organization understands both rare disorder research directions and emerging standards for clinical care. This level of understanding will facilitate the construction of programs that fulfill both scientific and patient needs. The benefits of strategic planning with your advisory board will help to set specific, focused goals for the community. This includes synchronizing research priorities with foundation programs. We have found that maintaining an open dialogue with our advisory can directly reshape basic research directions, while also stimulating discussions for setting standards for clinical care.