STEP 2

DEVELOP THE LANDSCAPE

OVERVIEW

Once you're ready and prepared, the next step is to understand the current situation through developing a landscape. The purpose of the landscapes is not to simply describe the external environment – it is vital to also frame the findings in a way that identifies where you are on the map and where are the gaps and unmet needs.

This results in hypotheses for the white space that can be filled by the unique capabilities of your particular cure-seeking organization.

This part of the toolkit describes a structured approach to secondary research to capture the state of science, translation, data and advanced analytics, clinical development, funding, clinical care, and outcomes for the disease.

The approach includes a consideration of the analysis approach. It's often useful to plot your findings on a two by two (2x2) matrix with axes designed to discriminate clusters of ongoing activity and isolate white space hypotheses for where your capabilities fill critical gaps in the landscape.

Without a hypotheses on how to target white space – the key opinion leader interviews will lack the specificity required to elicit actionable guidance.

Three Key Success Factors

1

A comprehensive perspective on the cure ecosystem and its adjacencies

It is often surprising to realize how broad the landscape must be to capture the full ecosystem insinuated in developing a cure. The following section outlines the cure value chain and the constituencies to study to comprehensively landscape your disease.

The universe includes academic medical centers, biopharma companies, venture capital, diagnostics and biobanking, advanced analytics, other disease foundations, and community hospital and health systems.

Three Key Success Factors continued

Academic Medical Centers (AMCs)	AMCs are hospitals with a tripartite mission of care, education and research. There are about 120 in the US, or about 5% of all US hospitals. Some pre-eminent examples include Mayo Clinic, Massachusetts General Hospital and UCSF Medical Center. Cures almost invariably start with fundamental science in AMCs that explores the root cause of disease and experiment with interventions in the pathway to interrupt development of the disease. It will be important to determine if the biology of your disease is understood and whether the science is supporting symptom treatment (management) or disease modification (cure).
Biopharma Companies	The best science gets bridged into ideas for therapeutics (translational science) and then into clinical research where the safety and efficacy of experimental therapies are evaluated. This research is sponsored by both academic centers through grants and the biopharma industry through sponsored clinical trials. It will be key to map out the status of therapies in the pipeline from pre-clinical translational science through first in human, proof of concept, large scale statistical efficacy, and finally, any approved therapies with their risks and benefits.

Three Key Success Factors continued

Venture Capital	The innovative early stage clinical research pipeline is often concentrated in venture funded start-up companies and then tends to transition to the pharmaceutical industry before marketing and reimbursement approval. Knowing where Venture is focused – and where the gaps are – may provide key ideas on the addressable white space.
Diagnostics and Biobanked Tissue	Increasingly, therapies are becoming specific to particular genetic or other molecular markers. Many therapies today incorporate a companion diagnostic to identify patients that will benefit. The activities of leading players in the biobanking of tissue and the development of esoteric assays and diagnostics can often provide clues into where the puck is headed for your disease (or analogues of your disease).
Analytics: Artificial Intelligence (AI) and Machine Learning (ML)	The advent of large scale, deeply characterized and longitudinal data has created the opportunity for AI and ML approaches to recognize unanticipated patterns and correlations between patient profiles, therapies and outcomes. As you develop the landscape it will be important to understand if these technologies are fit for purpose in your disease, if they have been commissioned and deployed - and if not if why and if that opens a white space opportunity.

Three Key Success Factors continued

The Not-For-Profit Universe	Given the grass-roots origins of many not-for-profit disease focused entities - it's possible (even likely) that your mission may overlap with existing organizations at either a national or international level. The landscape of potentially similar organizations will help identify areas to avoid duplication or find complementarity.
Community Hospital / Health Systems	Community hospitals provide the vast majority of care in the US and include innovators such as Intermountain Healthcare and Kaiser Permanente. These clinics deliver standard of care diagnosis and treatment. Your white space may involve optimizing treatment pathways through improving decision support or reducing the variance in standard of care. Some areas to map out would be: • How is a diagnosis made? • What is the standard of care and variance? • Are outcomes for approved therapies understood? • Are there screening methods in place to diagnose patients sooner?

Three Key Success Factors continued

2

An analysis plan that specifies how and where to get secondary data

Much of the data you will need to populate the landscape is captured in publicly available sources. The analysis plan should include the following:

Some key steps to prepare are:

- PubMed – scientific, translational and clinical research peer reviewed publications
- Clinicaltrials.gov – every clinical trial reported by Federal mandate
- Company websites and SEC filings – development programs and pipeline assets
- UpToDate – details on how disease is approached in the clinic
- NCCN – well established guidelines for treating specific cancers
- Professional organizations (American Association of Cardiologists, ASCO, etc.)

Three Key Success Factors continued

3

Facility with analytical approaches used to identify and communicate white space

Identifying white space matched to your mission and capabilities requires an analytical framework to capture and visualize secondary data.

For each strategy this can be an extensive exercise in exploring multiple views of the data to inform your hypotheses. However, if you're just starting out don't let the perfect be the enemy of the good. Here are three illustrative examples of variables that when plotted against each other in a 2x2 matrix can potentially identify immediate questions (like who to partner with) to more technical opportunities (like gaps in the funding or bio-specimens).

Simply capturing who the leading AMCs and biopharma companies are in your space on one axis and plotting against your perception of how willing they are to work you is a great way to identify potential partners.

Mechanisms of action (from the science) plotted against biopharma pipelines (from the industry) may identify that new science such as a vaccine or immunotherapy is locked in AMC studies and not translated to industry. Your hypothesis may be that this due to a lack of venture capital.

Emerging platform diagnostics (such as circulating free DNA assays or genetic markers) plotted against use in adjacent disease states may indicate that your cure is not represented. A hypothesis may be that this is driven by a lack of tissue.