

How allogeneic cell therapy developers can benefit from using a combination of CIBMTR® Bioinformatics Consulting, CIBMTR CRO Services and Cell Sourcing solutions from NMDP BioTherapiesSM

Situation

A client initiated a Phase I clinical trial for its allogeneic cell therapy. Several months in, limited success recruiting patients had led to an expensive lack of progress. The client needed guidance and support to get the clinical trial back on track.

The client wanted to understand geographic areas in the United States that had the highest density of patients that are heterozygous for a specific human leukocyte antigen (HLA) allele. That would allow it to select centers for its clinical trial in areas with more patients who fit the HLA profile, leading to a higher likelihood of patient recruitment on the trial. In addition, the client needed to estimate available donor pools in the U.S. that were homozygous or heterozygous for the same HLA allele.




The client engaged with the CIBMTR Bioinformatics Consulting team through NMDP BioTherapies. They aimed to identify U.S. states and counties with the highest density of patients and donors with the specified

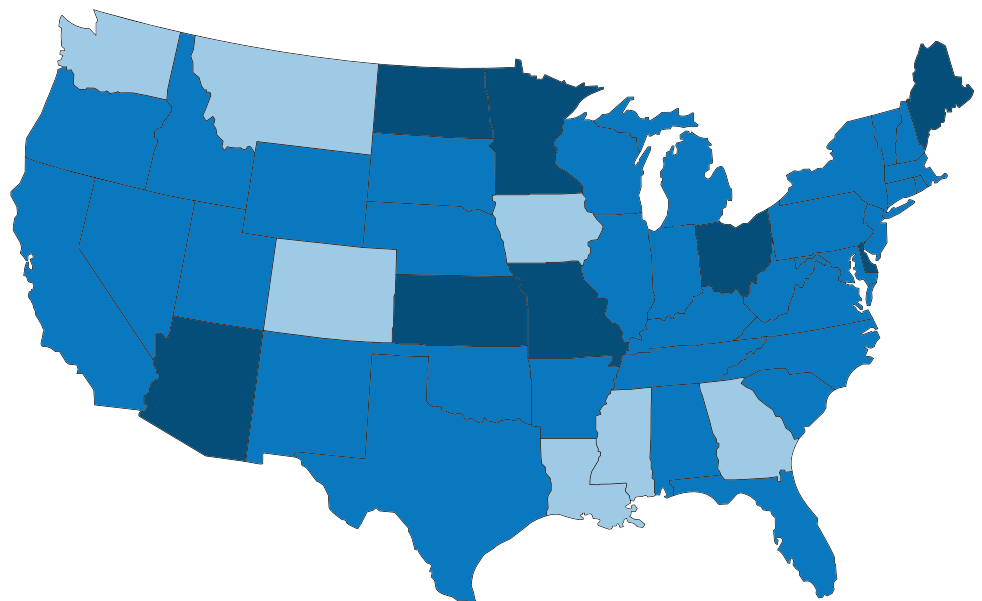
HLA profiles. In addition, they wanted to model match likelihoods and estimate available donors from multiple race groups to meet the demand of the target patient populations.

Approach and results

The Bioinformatics team used proprietary anonymized donor and patient geocoded data to identify states with the highest density of potential donor and patient pools.

Example of a density map of patients with a specific HLA profile by state

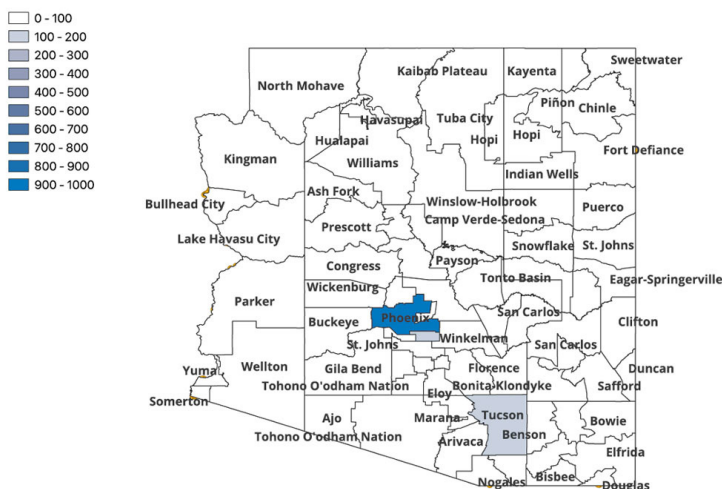
-  0.1 - 0.2
-  0.2 - 0.3
-  0.3 - 0.4



Approach and results (continued)

For the top states, the team then developed density maps at the county level to identify areas of potential recruitment as well as the population distribution of the requested HLA genotype.

Example counts of patients with a specific HLA profile by county in Arizona



In addition, through knowledge of match predictions and donor availability patterns, the Bioinformatics team estimated the expected number of matches for each patient. The team obtained these values from historical archived searches used to identify patterns of donor availability and match probabilities. It also used the current donor pool to estimate the actual number of donors available for each race group.

With this valuable information, the client was able to make data-driven decisions to get the clinical trial back on track. Had the client taken a different approach prior to initiating the clinical trial, the client could have saved substantial time and cost.

An alternate approach for allogeneic cell therapy developers

Clinical trial design and engagement strategies are key to successfully delivering clinical trial results.

NMDP BioTherapies offers a full suite of services that, when used together, can help cell and gene therapy clients plan, build and execute successful clinical trials.

If a client proactively leverages **CIBMTR Bioinformatics Consulting** services during clinical trial design, it has a clearer picture of where donors and patients with a therapy-specific HLA profile are located.

CIBMTR CRO Services can use the initial analysis to provide expertise and insights to help the client design the clinical trial. Through a direct link to the CIBMTR Outcomes database—which collects cell therapy and transplant population data for nearly all U.S. centers—the CRO can leverage HLA genotype sources against active patient populations to identify centers that can best target recruitment for clinical trials.

The **Cell Sourcing** team can use the donor pool analysis to refine its strategy as it recruits donors for the client.

By combining the available services, clients can save time and are more likely to achieve their clinical trial recruitment efforts.