Finding a New Use for Thalidomide in Multiple Myeloma Patients

In 2002, Cures Within Reach* funded Dr. Vincent Rajkumar at Mayo Clinic to repurpose thalidomide, a treatment for leprosy, for treating a rare blood cancer, multiple myeloma. His earlier studies demonstrated that thalidomide was an anti-angiogenic drug, as it prevented the growth of new blood vessels, a key mechanism in cancer cell growth.

In 2003, Dr. Rajkumar published the final results of his Phase II clinical trial, showing that thalidomide had significant potential as part of initial therapy for asymptomatic early-stage multiple myeloma to delay progression to symptomatic disease. Roughly one-third of the trial participants had a partial response to therapy (at least a 50% reduction of a multiple myeloma-linked protein) and nearly one-third more had a minor response (between 25-49% reduction). In addition, this was accompanied by improvements in bone marrow plasma cells. Within the first 24 months of his Phase II clinical trial, Dr. Rajkumar released strong enough results that physicians began prescribing thalidomide earlier in the course of multiple myeloma treatment.

* This was funded by Cures Within Reach’s founders, which became Partnership For Cures, now CWR.

KEY FACTS
- CWR provided $300,000 to fund Phase I/II clinical trials repurposing thalidomide for multiple myeloma

IMPACT
- Dr. Rajkumar published over 15 articles on these results
- The FDA approved thalidomide for multiple myeloma in 2006
- Further industry investment led to Revlimid, a more potent and safer class of treatments