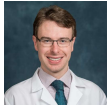


REPURPOSING OLD DRUGS AS NEW THERAPIES FOR METASTATIC THYROID CANCER

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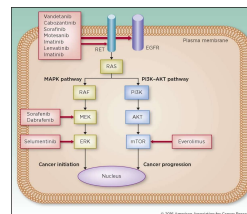
PROPOSED TREATMENT

Evaluate the efficacy of combining sirolimus and cyclophosphamide in patients with metastatic thyroid cancer.

Previous molecular and genomic analyses in thyroid cancer have demonstrated potential targets for repurposed therapeutics.

We looked at cancers with similar mutations in sarcoma and saw they responded to cyclophosphamide and sirolimus. We tried this combination in patients with aggressive thyroid cancer and anecdotally had success. Subsequently, we have published on the safety of the combination.

We propose building on this past experience and evaluating not only the combination's safety, but also efficacy in treating thyroid cancer.



This is of profound interest given the lack of current treatment options in the patient population and potential cost savings with this repurposed combination:

- Current Drugs: \$16-17k/month
- Our Combo: \$976/month

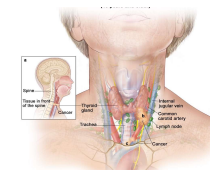
SUMMARY STATEMENT

Clinical trial evaluating the combination of cyclophosphamide and sirolimus in thyroid cancer to help an underserved population with few treatment options.

DISEASE/CONDITION

Thyroid cancer is a common malignancy. Despite aggressive treatment, approximately 15% of patients will develop incurable disease. In the US alone, approximately 9,400 patients are diagnosed each year.

- Increasing incidence and prevalence
- Tragically understudied given rarity compared to other common cancers, and resistance to most therapies employed in other malignancies
- Although targeted drugs are in development, there is a lack of trials for this incurable disease



CURRENT TREATMENT

Prior to systemic therapy, the mainstay of management is the combination of surgery and radioactive iodine (RAI) therapy.

Currently only two drugs, Sorafenib and Lenvatinib, have been shown to be beneficial for the treatment of RAI refractory thyroid cancer.

Treatment is associated with significant cost (\$16-17k/month) and side effects.

PROJECT

We are conducting a phase II study to evaluate the efficacy of cyclophosphamide and sirolimus in the treatment of patients with metastatic thyroid cancer.

We will enroll 19 patients with RAI refractory thyroid cancer to evaluate the combination of cyclophosphamide and sirolimus.

In addition to obtaining information about the response rate to cyclophosphamide and sirolimus, we will be collecting questionnaires to assess the impact of treatment on patients' quality of life.

Trial is currently open at the University of Michigan and rapidly accruing. Investigator funding has already provided \$102k (total budget of \$145k).

Funding would be used to support our research nurse and immediately facilitate increased enrollment and the ability to finish the trial sooner.

If this trial suggests benefit, we would have the ability to offer a novel therapy that is well tolerated and has significant potential cost savings.

