

WHO WE ARE: MISSION & VISION

Rafael Pharmaceuticals is a privately held, clinical-stage, rare cancer therapeutics company.

MISSION: To develop highly selective, well-tolerated and effective therapies for rare cancers that have few to no treatment options available, inaugurating a new era of hope.

VISION: We strive to be a high-growth, fully integrated leader in rare cancer therapeutics, focused on improving the lives of people with rare cancers.

CANCER METABOLISM STRATEGY

- Cohesive pipeline that targets the metabolic foundation for tumor growth, by hitting DNA replication inputs and mitochondria
- Targets metabolic processes specifically important to cancer cells
- Unique mode of mitochondrial targeting that is well tolerated with activity across wide variety of cancers
- Integrates the immune system
- Potential therapeutic options for a wide range of difficult to treat cancers
- Lead asset, devimistat, has shown to have a large safety profile, enabling use in chronic dosing and in combination with current standards of care with minimal added toxicity

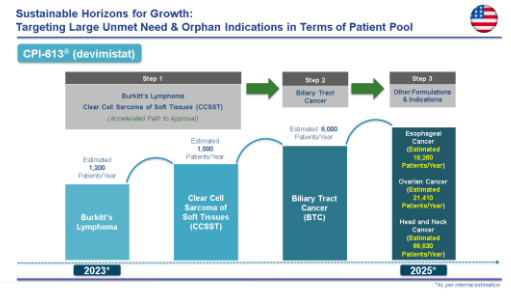
CPI-613® (DEVIMISTAT) & PIPELINE

- Our lead drug, devimistat, is being evaluated in clinical studies as a single agent, as well as in combination with standard drug therapies in patients diagnosed with rare solid tumors or blood cancers.
- Over 800 patients to date have received devimistat in more than 20 ongoing or completed clinical trials on multiple solid tumors and hematological malignancies.
- Devimistat has demonstrated positive efficacy signals in several clinical trials, including:

Indication	Unmet Medical Needs	Developmental Date
Relapsed or Refractory Burkitt's Lymphoma	<ul style="list-style-type: none"> ▪ Poor prognosis ▪ Treatment options remain undefined for patient who relapse after first-line ▪ Nearly all patients die of their disease, typically within weeks to a few months 	<ul style="list-style-type: none"> ▪ Devimistat monotherapy exhibited complete remission in two patients (which is extremely rare in relapsed or refractory setting) ▪ Enrollment in phase 2 study is ongoing
First Line Advanced Biliary Tract Cancer	<ul style="list-style-type: none"> ▪ Options for systemic chemotherapy remains limited ▪ Only a few meaningful improvements over the past few decades 	<ul style="list-style-type: none"> ▪ Devimistat in combination with gemcitabine and cisplatin exhibited 40% overall response rate (ORR) ▪ Enrollment in phase 2 portion of the study is ongoing

- Devimistat has been granted **US orphan drug designation** for Burkitt's lymphoma, biliary tract cancer, clear cell sarcoma, pancreatic cancer, acute myeloid leukemia (AML), myelodysplastic syndromes and peripheral T-cell lymphoma.
- Devimistat has been also granted **EU orphan drug designation** for Burkitt's lymphoma, pancreatic cancer and AML.
- Novel chemical class, composition of matter and formulation **protected to 2028** across the U.S., Canada, EU, Australia, Israel and major markets of Asia (China, Hong Kong, Japan, South Korea, Taiwan).

A Pipeline For Sustainable Growth



- Rafael has conducted over 20 ongoing or completed clinical trials in multiple hematological malignancies and solid tumors
- **Rafael initiated a Phase 1/2 clinical trial of devimistat in patients with relapsed clear cell sarcoma and completed dose escalation portion with no dose-limiting toxicity (DLT)**
- Successfully completed Phase 1 of Phase 1/2 clinical trial of devimistat with gemcitabine and cisplatin in patients with advanced biliary tract cancer. Phase 2 is ongoing.

EXPERIENCED LEADERSHIP TEAM

Sanjeev Luther, President & CEO

Robert G. L. Shorr, PhD, DIC, Chief Scientist/Co-Founder

Timothy S. Pardee, MD, PhD, Chief Medical Officer

Jenna Vadas, Head, Finance

Priya Marready, Head, Clinical Operations

Mike Stelmah, VP, CMC, QA/CQA

LEADING ADVISORY BOARD MEMBERS

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