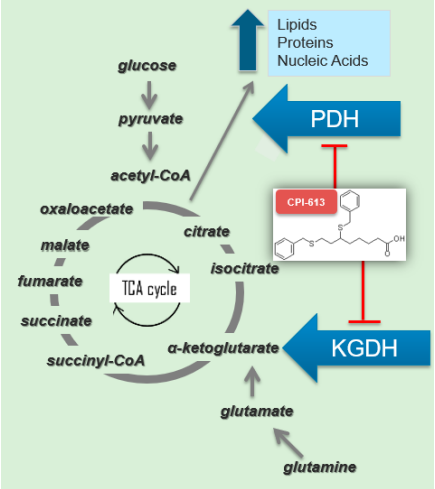
Privately held, clinical-stage, metabolic oncology therapeutics company, focused on developing cancer metabolism-based drugs

*Our **Mission** is to develop innovative, highly selective, well tolerated and highly effective anti-cancer agents by selectively targeting altered metabolism in cancer cells. Our immediate goal is to improve the quality of life of patients with Pancreatic Cancer, which is the deadliest cancer worldwide with very limited treatment options.*

*Our **vision** is extending and enhancing the lives of patients with Gastrointestinal (GI) Cancers.*



- Cancer cells extensively reconfigure normal cellular metabolism
- Our Altered Metabolism Directed (AMD) platform targets altered regulation of metabolic processes specific to cancer cells
- There are two major re-regulated control points which regulate carbon influx into Tricarboxylic Acid (TCA) cycle of cancer cells
- These are Pyruvate Dehydrogenase (PDH) which inputs pyruvate carbon and Alpha-Ketoglutarate Dehydrogenase (KGDH) which inputs glutamine-derived carbon
- CPI-613 selectively blocks PDH and KGDH (Multi Targeted Approach), by a distinct proximate mechanism, triggering cell death
- AMD platform is highly specific, simultaneously attack multiple targets, minimally toxic and have broad spectrum activity across wide variety of cancers. It is a potential therapeutic option for difficult to treat cancers. Because of very low toxicity, it can be used in chronic dosing and also in combination with current standards of care with minimal additional toxicity.

Intellectual Property:

CPI-613 is a novel chemical class, compositions of matter and formulations protected until 2028 across US, Canada, EU, Australia and major markets of Asia (China, Hong Kong, Japan, South Korea, Taiwan)

Clinical Studies & Pipeline:

- Our lead molecule CPI-613 is currently being evaluated in 15 Phase I to Phase II trials as a single agent, as well as in combination with standard drug therapy for hematological malignancies and solid tumors
- To date, over 300 subjects have received one or more doses of CPI-613 and the drug exhibited very good signal of efficacy with excellent response rate and extended duration of response in several tumor types
- In Pancreatic Cancer, CPI-613 in combination with modified FOLFIRINOX exhibited objective response rate of 61%, median overall survival of 19.7 months and median progression free survival of 9.9 months
- In elderly patients with Acute Myeloid Leukemia (AML), CPI-613 in combination with high dose cytarabine and mitoxantrone exhibited 48% complete remission
- In both these trials, the efficacy of CPI-613 combinations were substantially higher than standard therapy.
- In Peripheral T-cell Lymphoma (PTCL), CPI-613 in combination with bendamustine exhibited 86% objective response rate
- CPI-613 also showed very good safety profile both as single agent and in combination with other standard-of-care drugs
- CPI-613 has been granted U.S. Orphan Drug Designation for Pancreatic Cancer, AML, Myelodysplastic Syndrome (MDS), PTCL and Burkitt's Lymphoma

Future Directions: Rafael is planning to initiate company sponsored randomized phase III trial in Pancreatic Cancer and AML in 2018 second half and these trials are expected to be completed by 2021. Rafael's primary target is to successfully complete these 2 pivotal trials and launch CPI-613 in market by 2021 – early 2022. In addition to that, Rafael is also focused in developing next generation molecules in AEMD platform, develop oral form of CPI-613 and develop these drugs in combination with immune-oncology medications.

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