Client

Finding New Niches and Growth Areas in Specialized Testing

Juniper
LIFESCIENCES

Section 1: Background

Client

Client is a leading diagnostic laboratory that specializes in complex antibody testing. Client is one of two commercially available L-asparaginase assays in the world and the only one that offers asparaginase antibody assays. The assay determines the enzyme activity of L-asparaginase or the presence of anti-asparaginase antibodies in ALL patients who have been treated with Asparlas®, Oncaspar®, Rylaze®, and Erwinaze®. The L-asparaginase activity test supports therapeutic drug monitoring and helps physicians identify patients experiencing "silent inactivation."

As part of their growth goals, the client would like to identify new opportunities in the hematology-oncology diagnostics market for the highly specialized testing they perform. Client currently has a strong presence in the childhood and adult leukemia markets.

However, Client would like to explore other potential markets such as:

Testing for hospital-based treatments

Client prefers to provide more testing for hospital-based oncology treatments, which have better compensation schemes than outpatient testing. The client currently performs L-asparaginase assay testing for more than half of the children's hospitals in North America. Client believes it has the technical capacity and expertise to develop more assays for medications or antibodies to those medications.

Other targets

Client has previously worked to develop antibody testing for pharmaceutical companies, including Jazz Pharmaceuticals. They would like to explore more opportunities in this space. Pharmaceutical companies could benefit from this type of testing during their drug development process within their oncology franchises.

- 9,280 new cases of CML are estimated to occur in the U.S. in 2024 (American Cancer Society).

There are several subtypes for each main type of leukemia, based on specific characteristics of the cancer cells. Understanding the subtype is important for determining the most appropriate treatment approach.

See ***Appendix 1*** for more information on the many subtypes of leukemia.

Leukemia Treatment

Treatment for leukemia depends on various factors, including the type and subtype, the patient's age and overall health, and whether the leukemia has spread to other parts of the body. Common treatments include chemotherapy, targeted therapy, radiation therapy, immunotherapy, and stem cell transplantation. The goal of treatment is to destroy leukemia cells, prevent the spread of the disease, and allow normal blood cell production to resume.

Asparaginase is an enzyme that plays an important role in cancer treatment, particularly in the management of leukemia. It works by breaking down the amino acid asparagine into its components, aspartic acid and ammonia. In leukemia treatment for ALL, cancer cells require large amounts of asparagine to grow and survive. They cannot produce it on their own so they rely on obtaining it from the bloodstream. Asparaginase therapy exploits this dependency by depleting circulating asparagine levels. By breaking down asparagine in the bloodstream, asparaginase effectively starves leukemia cells of this essential nutrient, causing them to undergo apoptosis, or programmed cell death. Normal cells can produce their own asparagine, so they are less affected by the depletion caused by asparagine treatment. However, some normal tissues, such as the liver, rely on circulating asparagine to some extent, so side effects such as liver toxicity may occur (Trimpont 2022).

See ***Appendix 2*** for more information on the types of leukemia treatment available.

Section 3: Market Analysis

Market Considerations Overview

1. Asparaginase

Asparaginase is a vital component of first-line therapy for patients with ALL, indicating potential for market growth of the drugs and L-asparaginase assays and antibody tests (Brumano 2018). The demand for L-asparaginase assays and antibody tests is expected to increase in the coming years. The high cure rates achieved with L-asparaginase therapy in pediatric ALL and the importance of asparaginase level assessment suggest a growing need for these tests (Trimport 2022).

Market Size

In 2022, the asparaginase market was valued at USD 667 million. It is expected to grow with a CAGR of 16% by 2023 (UnivDatos). Research is ongoing to expand its application contributing to its market growth. It is being explored in nanomedicine and biomedical engineering, among other fields. Various governments and asparaginase producers and manufacturers are investing in research and development, which is expected to also increase market growth in the coming years (UnivDatos).

The development of novel biomarkers and the integration of artificial intelligence in diagnostic testing are expected to drive the market growth. Additionally, the growing focus on personalized medicine and the need for early diagnosis will further contribute to the expansion of this industry (Trimpont 2022).

Product-Market Fit

By adding ELISA assays to monitor other Leukemia treatments such as Blincyto or Besponsa, Client could become more competitive in the lab testing market while creating a niche that does not seem to be occupied by the larger reference lab companies. Adding an assay for Adcetris expands into the Lymphoma space, but still remains targeted within Hematology-Oncology. This strategy would allow Client to remain lean in Sales and Marketing while increasing business by calling on the same physicians.

Methodology

Primary research

Experts in Hematology-Oncology were recruited for this study using LinkedIn, Email reach out, personnel connections, and referrals from expert interviews. Interviews with experts were conducted using a discussion guide developed following a review of the literature regarding potential new market opportunities. The discussion guide was reviewed and approved by Client Labs prior to conducting the interviews. All interviews were conducted using Zoom and recorded with the permission of the experts, and are available for viewing.

Recruiting in this market was challenging given the very niche specialization of the physicians that were interviewed. Multiple physicians replied to the interview request, but were screened out of participation based on the patients they treated.

Note: Interviews became more specific and targeted as information was gained from the initial interviews. A different set of physician experts will be needed to explore opportunities in areas such as immune-mediated diseases.

Secondary research

This literature review on leukemia assays was conducted beginning in April 2024 using the following biomedical databases: 1) the National Library of Medicine's PubMed, 2) Medline, 3) CINAHL Plus, 4) Embase, and 5) Cochrane. For clinical decision-making guidelines, we consulted tools such as UpToDate and StatPearls. Additionally, we queried market data using ProQuest.

Where appropriate, we also consulted relevant textbooks, news articles, official communications from regulatory agencies (e.g., FDA), and national and international society guidelines.

Limitations

We acknowledge certain limitations such as:

- **Limited sample size:** Best efforts were made to recruit 10 Hematology-Oncology experts however the total number of interviews for this project was 8. Juniper noted that the experts in this field were extremely specialized, even with the types of Leukemia they treat. This specialization made it more difficult to explore other areas of interest such as Lymphoma and Immune Mediated conditions as most experts did not see patients outside of their specialization. It is recommended that further exploration is conducted with other specialists to determine if other areas of testing may represent opportunities for Client.

- **Limited evidence:** In producing this report, we have considered the best available evidence. However, because there are certain gaps in knowledge until more information is available, certain recommendations are based on societal guidelines or expert opinions. Expert recommendations do not replace high-level evidence, particularly randomized controlled trials and systematic reviews and metanalyses.58 Given this limitation, the reader should exercise caution when using the information contained in this report to make clinical or business decisions.

Authors:

~ Michael Solomon, MPH, MBA
~ Donna Taraborelli, DrPH, MPH, MBE
~ Samuel Sarmiento, MD, MPH, MBA
~ Carrie Haney, MSPH

Juniper Life Sciences
San Diego, CA

Appendices

1. *Types of Leukemia*

Leukemia Type	"Incidence U.S. 2024"	Description	Subtypes
Acute Lymphocytic Leukemia (ALL)	~6550	• ALL is most common in children. Males at higher risk • ALL starts in the lymphoid cells of the bone marrow. • ALL often spreads quickly to the blood in other parts of the body, such as: CNS. Lymph Nodes, Liver, Spleen, Testicles."	• B-cell ALL • T-cell ALL • Philadelphia chromosome-positive (Ph+) ALL • Mixed phenotype acute leukemia (MPAL)"
Acute Myelogenous Leukemia (AML)	~28000	• AML is an aggressive leukemia in adults. It can also affect children. • AML starts in the myeloid cells of the bone marrow and can spread quickly into the blood. • From there, AML can spread to: CNS. Lymph Nodes, Liver, Spleen, Testicles."	• "AML with recurrent genetic abnormalities • AML with t(8;21)(q22;q22.1, • AML with inv(16)(p13.1q22), t(16;16)(p13.1;q22), • AML with t(15;17)(q22;q12) • AML with myelodysplasia-related changes • AML not otherwise specified (NOS) • Therapy-related AML • Secondary AML"
Chronic Lymphocytic Leukemia (CLL)	~20700	• CLL is the most common type in adults, particularly older adults. • CLL affects lymphoid cells and typically progresses slowly."	• "CLL with typical immunophenotype • CLL with atypical immunophenotype • CLL with mutated IGHV genes • CLL with unmutated IGHV genes"
Chronic Myelogenous Leukemia (CML)	~9280	• CML occurs mainly in adults. • CML affects myeloid cells and typically progresses slowly."	• CML in chronic phase • CML in accelerated phase • CML in blast phase"

References

1. About Acute Lymphocytic Leukemia (ALL). American Cancer Society. Accessed June 4, 2024. https://www.cancer.org/cancer/types/acute-lymphocytic-leukemia/about.html.

2. Acute Myeloid Leukemia (AML) in Adults. American Cancer Society. Accessed June 4, 2024. https://www.cancer.org/cancer/types/acute-myeloid-leukemia.html.

3. Chronic Lymphocytic Leukemia (CLL). American Cancer Society. Accessed June 4, 2024. https://www.cancer.org/cancer/types/chronic-lymphocytic-leukemia.html

4. Chronic Myeloid Leukemia (CML). American Cancer Society. Accessed June 4, 2024. https://www.cancer.org/cancer/types/chronic-myeloid-leukemia.html

2. *Types of Leukemia treatment*

Generic Name	Brand Name	Manufacturer	Cancer Type Treated
Chemotherapy			
vincristine, liposomal vincristine	Marqibo Vincasar PFS	Talon Therapeutics Teva Therapeutics	ALL subtype
daunocrubicin + cytarabine doxorubicin	Vyxeos Lipidox	Jazz Pharmaceuticals Sun Pharmaceuticals	ALL
cytosine arabinoside, ara-C	aka Cytarabine	Sigma-Aldrich	"ALL, AML. Non-Hodgkin's Lymphoma"
L-asparaginase, PEG-L-asparaginase, pegasparagase	Asparlas Oncaspar Rylaze"	Servier Pharmaceuticals Servier Pharmaceuticals Jazz Pharmaceuticals"	ALL, LBL
Mercaptopurine (6-MP)	Purixan	Rare Disease Therapeutics	ALL; Blood, bone, breast, head and neck
Methotrexate	Otrexup (PF) Xatmep Rasuvo	Antares Pharmaceuticals Azurity Pharmaceuticals Medexus Pharmaceuticals	ALL
Cyclophosphamide	Cytoxan (generic)	Baxter Healthcare	ALL; Hodgkin's Lymphoma
Nelarabine	Arranon	GlaxoSmithKline	T-ALL; T-LBL
Immunotherapy			
Blinatumomab	Blincyto	Amgen	ALL
Inotuzumab ozogamicin	Besponsa	Pfizer	B-cell precursor ALL
Rituximab	"Rituxan MabThera"	Biogen Roche	Adults with CLL
Alemtuzunab	Lemtrada	Sanofi Aventis	B-cell precursor CLL
Gemtuzumab-ozogamixin	Mylotarg	Pfizer	AML
CAR-T Therapy			
Brexucabtagene autoleucel	Tecartus (brexu-cel)	Kite Pharmaceuticals	ALL
Tisagenlecleucel	Kymriah	Novartis	B-cell precursor ALL refractory; second stage or relapse
Tyrosine Kinase Inhibitors			
Imatinib	Gleevac	Novartis	Ph+ ALL; CML
Dasatinib	Sprycel	Bristol-Myers-Squibb	Ph+ ALL; CML
Nilotinob	Tasinga	Novartis	Ph+ ALL; CML
Ponatinob	Iclusig	Takeda	Ph+ ALL; CML
Bosutinib	Bosulif	Pfizer	Ph+ ALL; CML

Source: Manufacturer websites. Note: This is not an exhaustive list.

1926 Third Ave | San Diego, CA 92101 | juniper.life