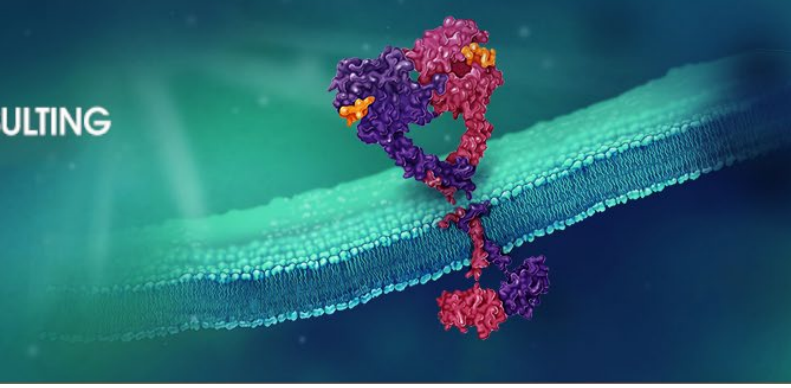
Case Study



INTERNATIONAL MARKET ACCESS CONSULTING

Where expertise drives strategy

Development of a Health Technology Assessment (HTA) Dossier for the National Institute and Care Excellence (NICE) and HTA Submissions in Select European and Canadian Markets

NICE National Institute for Health and Care Excellence

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Scope of Work and Deliverables (1)

IMAC partnered with our client to fulfil payer requirements in Europe and Canada. Our team of market access experts assisted the client by developing compelling HTA/reimbursement dossiers. The value of the therapy was presented in an accurate, succinct, and engaging story format with focus on overcoming payer objections.

The project began with a formal kick-off meeting where the client and IMAC exchanged relevant project-related information and discussed and agreed upon the framework for the assignment.

NICE HTA Dossier

IMAC provided the following services as part of the development of the NICE HTA dossier:

- At the start of the project, IMAC worked with the client to develop a strategy and timeline for the writing and review of the NICE HTA dossier sections.
    - The strategic plan took into account any outstanding assets (eg, the CE model if under development) to maximize efficiency.
    - Writing of NICE HTA dossier sections was in accordance with the dossier template and requirements.
    - Simple tables and graphs were created in a consistent style.
    - IMAC prepared an EndNote library and reference package that accompanied the submission.
    - IMAC incorporated comments following the client reviews.
- Development of a target product profile with value messages relevant to the UK market which were leveraged within the NICE HTA dossier.
    - In collaboration with the client, IMAC developed key messages (leveraging the Global Value Dossier) which served to structure the value story for the product in the NICE HTA dossier.
- Targeted literature searches to develop the following sections:
    - Epidemiology and clinical burden of the disease (leveraging any cross-over data from the economic models)
    - Treatment guidelines and clinical pathway for patients
    - Previous economic evaluations of relevance to the current assessment

imarketaccess.com
imac@imarketaccess.com
European Office:
+44 7748 742 540
North American:
+1 514 294 4016

Scope of Work and Deliverables (2)

HTA Submissions in Select European Countries

Based on the completion of the UK NICE HTA dossier, IMAC developed HTA submissions in select European countries as requested by the client. According to the resources available from the client at the time of initiation in each new country, IMAC worked with the client to determine a customized workflow plan for the HTA dossier. The timelines and budget reflected the substantial leveraging of content developed for the UK NICE HTA dossier developed by IMAC.

Overall Project Management

This project also included:

- Project management (ie, scheduling of deliverables and review cycles, invoicing, and regular project updates).
    - IMAC managed all communication with relevant client personnel who were reviewing the submission.
    - A detailed email accompanied each deliverable or follow-up after a web conference indicating the file that was reviewed, the date by which comments were requested, and the status of the document (ie, first review, second review, final review).
    - All team action tasks were clearly detailed and easy to follow.
- Meetings (including agenda and minutes) with relevant client staff.

Project Outcomes

NICE issued a positive recommendation for the client's therapy. Additionally, the therapy has been accepted or is currently under negotiation for reimbursement in all countries for which IMAC produced submissions, including:

- **Scotland**
- **Wales**
- **Ireland**
- **Finland**
- **Norway**
- **Sweden**
- **Portugal**
- **Belgium**
- **Canada**
- **Quebec**

INTERNATIONAL MARKET ACCESS CONSULTING

Related Experience

IMAC has competence across a wide range of therapeutic areas and has specific expertise with oncology. IMAC has considerable experience developing submissions to NICE for products in many therapeutic areas. Recently, IMAC developed a Highly Specialised Technology (HST) submission for an innovative gene therapy for an ultra-rare disease and Single Technology Appraisal (STA) submissions for a first-line oncology product and a first-in-class monoclonal antibody. Additionally, IMAC has supported strategic global HTA submissions for products in Europe and North America.

"We've been running on repeat business for the last 12 years. We know how to really build a strong story that links both clinical & economic aspects of your therapy together"
– Louise Perrault, President & CEO

"IMAC consists of a team of people that are experienced in not only developing submissions, but also in rescuing submissions that are struggling for different reasons. We are able to quickly assess the challenge(s), strategise a plan for optimising the value proposition of a product, and continue our support throughout the submission process. Because we are a team of experts, we are able to work quickly under tight deadlines that are often challenging for larger firms. We produce the highest quality products, on time and on budget, and we have an excellent success rate."
– Nicole Tunstall, Senior Consultant, HTA and Medical

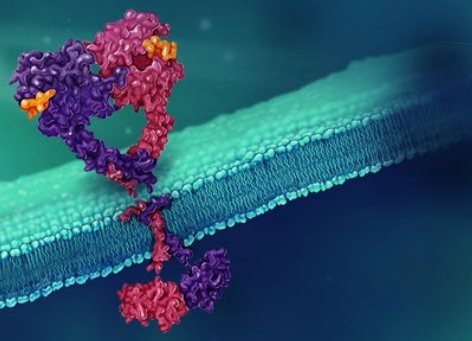
"We are people with experience, when we speak with our clients, we understand what you're talking about. We know how to develop products that stand up to review because of our expertise."
– Veronique Lauzon, Senior Health Economist

"IMAC is made up of more experienced qualified consultants who understand how to deal with the challenges many companies face when seeking market access for a new therapy. Because of this everything is completed on time, to a high level of quality, and a very good success rate."
– Eva Tsakonas, Senior Epidemiologist, Health Economist

"IMAC is above all a company of experts who are concerned about quality service throughout the duration of your project. We are a team of competent professionals who listen to and care about your needs and will successfully see your products through various evaluation processes while respecting the deadlines imposed."
– Ange Christelle Iliza, Research Assistant

"Working with IMAC is like adding a team of experts to your company for the duration of the project. From the project initiation to the final follow-up, you deal with the same experienced consultants who are there to advise and optimise the project whether it be an economic model, systematic literature review, HTA submission, global value dossier, manuscript, or conference presentation."
– Sarah-Gabrielle Béland, Senior Pharmacoepidemiologist

Background



CML is a proliferative disorder of the haematopoietic stem cells that results in dysregulated production of myeloid white blood cells.[1] CML is a rare cancer, accounting for 15% of adult leukaemias.[2] The disease course of CML progresses across three distinct phases: the initial indolent chronic phase (CP-CML), an intermediate accelerated phase (AP-CML) that lasts for less than 1 to 1.5 years, and an aggressive blast phase (BP-CML) that is usually fatal within 3 to 6 months.[1] In the absence of treatment, the prognosis of CML is poor, with expected survival of 3 to 5 years from diagnosis.[1] Current treatment options are based on the use of tyrosine kinase inhibitors (TKIs), allogeneic stem cell transplantation (allo-SCT) for suitable patients, and best supportive care (BSC)/palliative care. Despite significant advances in CML therapy following the introduction of first-generation (1G; imatinib) and second-generation (2G) TKIs (dasatinib, nilotinib, or bosutinib), a substantial proportion of patients continue to develop resistance to or intolerance of 1G and 2G TKIs.[3]

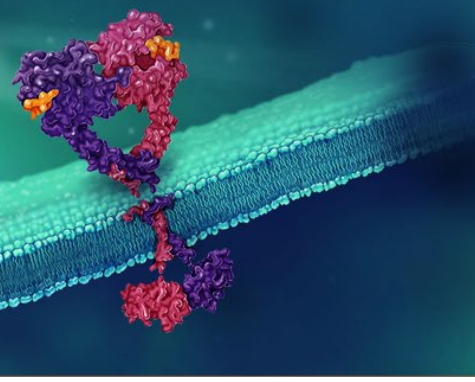
ALL is an aggressive lymphoproliferative malignancy that represents approximately 20% of adult leukaemias. The majority ALL cases show chromosomal and genetic abnormalities, and approximately 25% of adult cases of ALL have the Philadelphia (Ph) chromosome.[4] Ph+ ALL has an extremely poor prognosis. Prior to the advent of TKI, the median survival time of Ph+ ALL patients was 8 months, with only 10% of patients achieving prolonged remission with conventional chemotherapy alone.[5,6] Overall survival (OS) is substantially reduced with increasing age. Adults with Ph+ ALL have the poorest prognosis, with 5-year OS rates of 24.1% for those between 40 and 59 years of age, and just 17.7% for those between 60 and 69 years of age.[4] Even with currently available 1G and 2G TKIs, among patients resistant to and/or intolerant of prior therapy, survival is only 6 to 9 months.[7]

Patients with imatinib-resistant CML or Ph+ ALL who fail therapy with a 2G-TKI have limited treatment options and significant unmet medical need.

International Market Access Consulting (IMAC) was invited by a client, to submit a proposal describing the steps and budget required to develop a NICE HTA dossier and HTA submissions in select European countries for a new TKI therapy to address the significant unmet need in these patient populations. This document describes the steps required to complete this project.

---

[1] Kantarjian HM, Deisseroth A, Kurzrock R, *et al*. *Blood.* 1993;82(3):691-703.
[2] National Comprehensive Cancer Network (NCCN). NCCN Clinical Practice Guidelines in Oncology: Chronic Myelogenous Leukemia. Version 1.0: NCCN; 2016.
[3] Santos FP, Kantarjian H, Quintas-Cardama A, *et al*. *Cancer J*. 2011;17(6):465-76.
[4] National Comprehensive Cancer Network (NCCN). NCCN Clinical Practice Guidelines in Oncology: Acute Lymphoblastic Leukemia (ALL). Version 2.0: NCCN; 2015.
[5] Fielding AK. *Hematology Am Soc Hematol Educ Program.* 2011:231-237.
[6] Lee HJ, Thompson JE, Wang ES, *et al*. *Cancer.* 2011;117(8):1583-1594.
[7] Lilly MB, Ottmann OG, Shah NP, *et al*. *Am J Hematol*. 2010;85(3):164-70.



Expertise and Achievements

imarketaccess.com
imac@imarketaccess.com
European Office:
+44 7748 742 540
North American:
+1 514 294 4016