Market Opportunity Assessment for Immune Thrombocytopenic Purpura (ITP) - an Autoimmune Disease using the Commercial Forecasting Modelling

By

Brijesh Kanjani

Dissertation submitted for the partial fulfillment of the

MBA Pharmaceutical Management

Academic year, 2018-2020

The IIHMR University, Jaipur

1, Prabhu Dayal Marg, Sanganer, Airport
Jaipur 302029, Rajasthan
Ph: 0141 3924700, Fax: 3924738
Website: **www.iihmr.edu.in**

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PharmaACE

The certificate is awarded to

Brijesh Kanjani

In recognition of having successfully completed his Internship in the department of

Forecasting

and has successfully completed his Project on

Market Opportunity Assessment for ITP using the Commercial Forecasting Modelling

starting from

February 03, 2020 to May 03, 2020

at

PharmaACE Analytics LLP

He comes across as a committed, sincere & diligent person who has a strong drive and zeal for learning

We wish him all the best for future endeavors.

Dinesh Pandey

Training & Development

Zonal Head-Human Resource

Blueridge Qubix, SEZ, IT-5, Ground Floor, Phase 1, Hinjewadi, Pune – 411057
www.PharmaACE.com

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BRIJESH KANJANI

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COMPANY PROFILE

PharmaACE Analytics Pvt. Ltd. is life sciences focused company. Headquartered in USA, the company provides analytic solutions for efficient commercialization of pharmaceutical products.

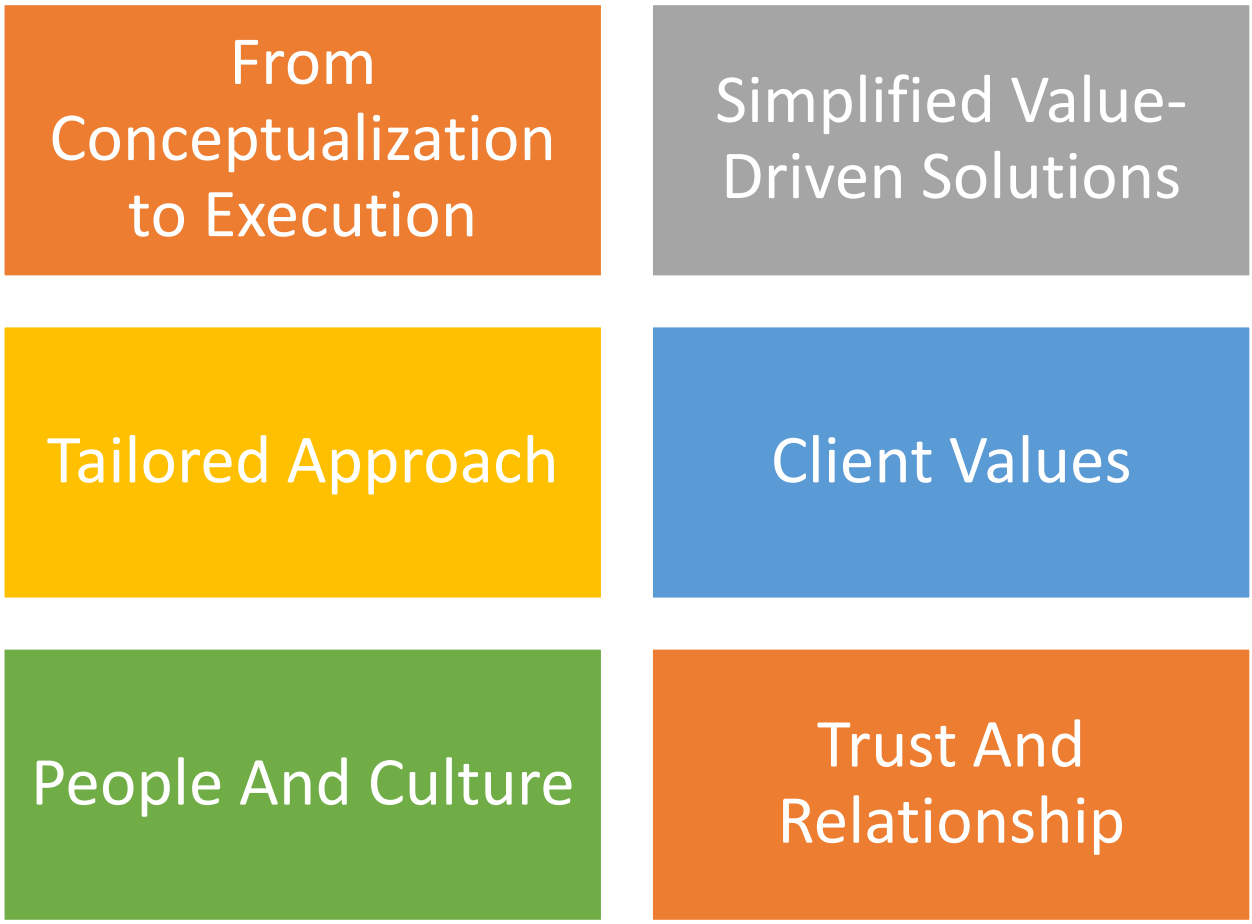
The company works through teams such as Competitive Intelligence, Forecasting and IT to provide pharmaceutical companies with business-critical research and insights that helps them to make informed decisions. The decisions enable the clients to develop strategies for sales and marketing of newly launched products, develop a perspective of the existing players, their strengths and weaknesses, opportunities for a particular product in the market, analyzing the threats and finding out ways to overcome it.

The **Competitive Intelligence team** is involved in conducting secondary search to answer various business questions for clients such as opportunities for a product in a market through pipeline assessment.

The **Forecasting team** is primarily involved in estimating a product's market share and revenue. It includes various data analysis techniques to collect and forecast sales and volume data to achieve a final revenue forecast.

Apart from these teams, the **IT team** is responsible for providing the support in terms of creating models so that the forecasting team can derive revenues by applying all the factors responsible for increase in revenue or decline.

The company stands out in the field of business consulting because of the differentiators like –



INTRODUCTION

Forecasting refers to the process of estimating a product's revenue and patient numbers. This helps the pharmaceutical companies to develop strategies to maximize their revenue and market share by considering the factors (launch of competitors' product, discount schemes etc.) that may lead to loss in revenue.

The time horizon of forecast ranges from:

- Monthly (12-36 Months)
- Annual (1-3 Years)
- Short-Term (3-5 Years)
- Long-Term (5—15 Years).

The major challenges associated with forecasting are inaccuracy, bias, too detailed or too general forecast. The key assumptions used in forecasting decide the accuracy of forecast and thus uncertainties around these assumptions lead to inaccurate forecast. Bias in forecast is unavoidable but hidden bias results in sub-optimal decision making.

The balance between a very general or detailed forecast is difficult to strike. An example of a general forecast is when the patient numbers are simply multiplied by market share of the product and then by price of the therapy to obtain revenue. This approach is valid but when questions like whether patient segmentation exists, compliance and testing rate are answered then the forecast loses its validity.

AIM

The aim of this study report is to gather in-depth understanding of an autoimmune disorder known as ITP (Immune Thrombocytopenic Purpura) including but not limited to the pathophysiology, treatment algorithms, prevalence & incidence across different markets This information was used to come up with a forecast flow & estimate the market potential of a pipeline product.

RESEARCH OBJECTIVES

To understand the definition and pathophysiology of the autoimmune disorder-ITP

To understand the developed treatment algorithms of the disease as per the US, European and Japanese guidelines.

To record and calculate the epidemiological considerations (incidence and prevalence) of the disease in 7 major markets.

To Create a proposed patient based forecast flow for the disease

RESEARCH METHODOLOGIES

Study Area - The study was conducted at PharmaACE Analytics Pvt. Ltd. Pune, Maharashtra with Immune Thrombocytopenic Purpura as the disease of interest

Study Design - It is an analytical study conducted for the ITP patients who are the potential customers for product X of the client using forecasting model. The market share, volume and revenue of the product were forecasted

Study Duration - The study was carried for 3 months from 3 February 2020 to 3 May 2020

Sample Size - The entire ITP patients of 7MM (US, UK, France, Italy, Germany, Spain, and Japan) were considered for estimation of volume and revenue of Product X. However various eligibility cuts like age and treatment rate were applied to arrive at the treated patient pool

Data collection methods - The epidemiological data for the 7MM around the globe was collected using the various source. A through secondary analysis of the various studies available on the disease was done with available secondary sourced literature, PubMed, Science direct, and other online resources.

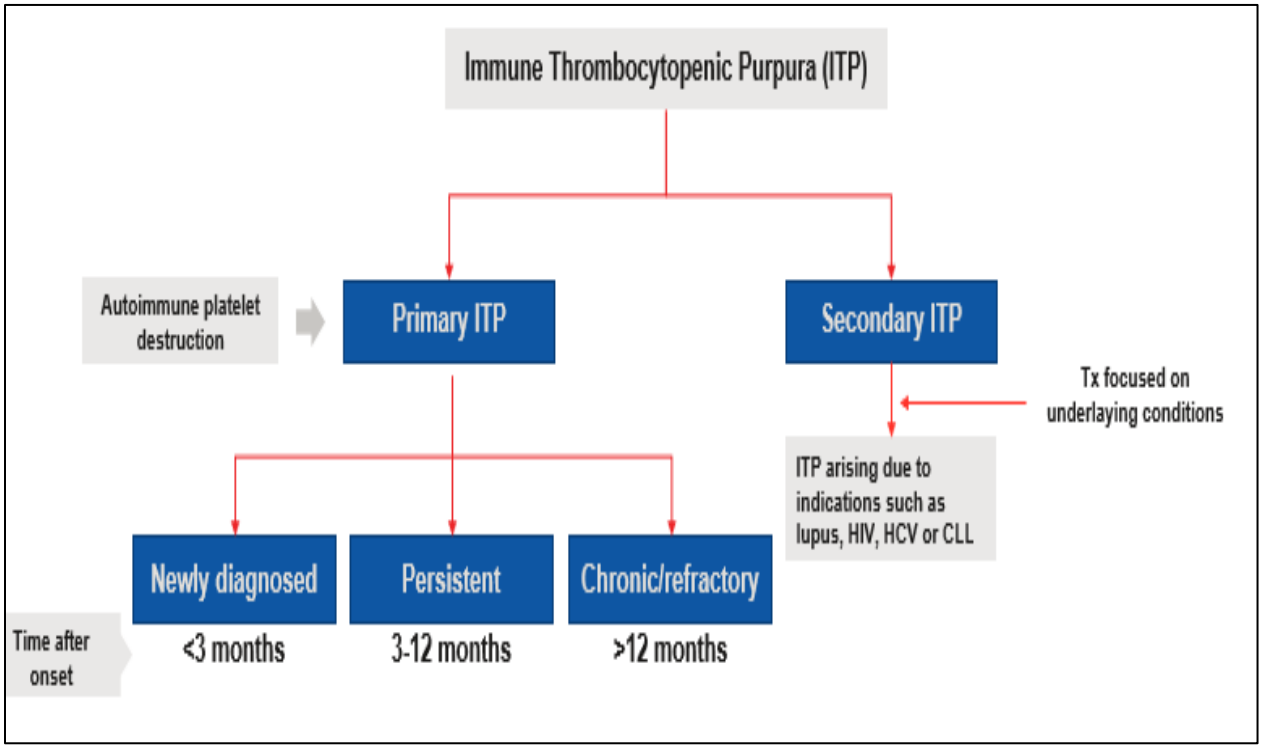
UNDERSTANDING THE DISORDER

Immune Thrombocytopenic Purpura (ITP) is an autoimmune disorder in which the patient's immune system reacts with a platelet autoantigen(s) resulting in thrombocytopenia due to immune-mediated platelet destruction and/or suppression of platelet production.

In the diseased condition, platelet membrane proteins, produce autoantibodies and cytotoxic T cells. The initial antigenic response probably occurs in the spleen followed by stimulation of other antibody-producing tissues

Under the diseases condition the produced platelet are destructed or the production of new platelets is suppressed.

In summary, activation of the immune system by platelet autoantigens in ITP may result in platelet destruction and/or inhibition of platelet production. The mechanism likely to vary in each of the individual patients.



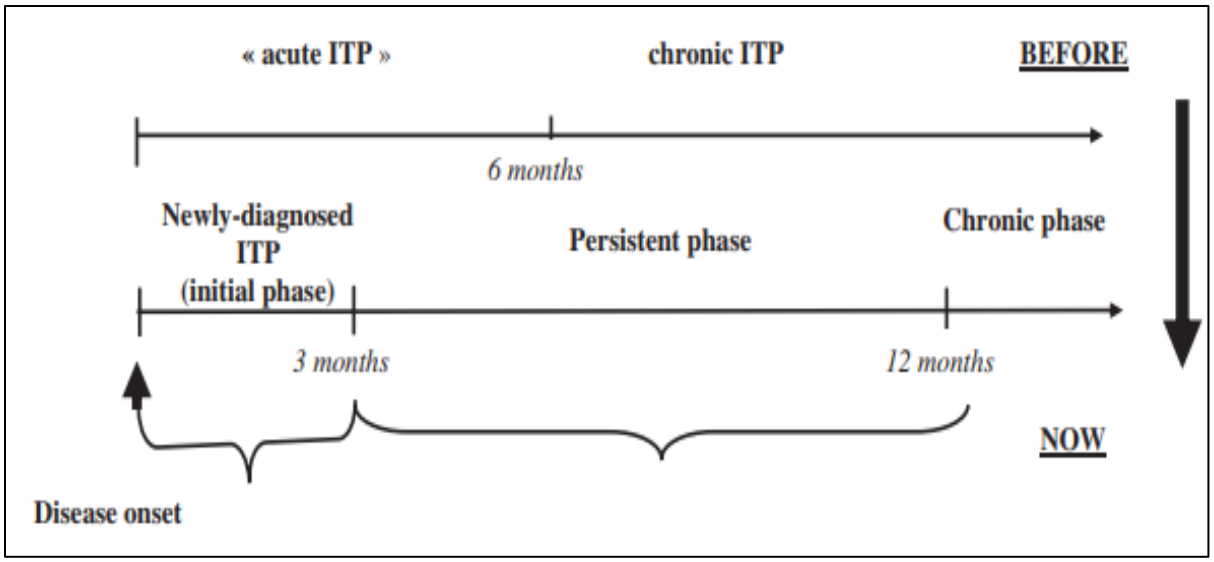
DIAGNOSIS

The principal diagnosis of ITP is based on the physical examination, history, CBC, and the peripheral smear examination. The diagnosis such made should eliminate the other causes of thrombocytopenia.

Classification of the Disease

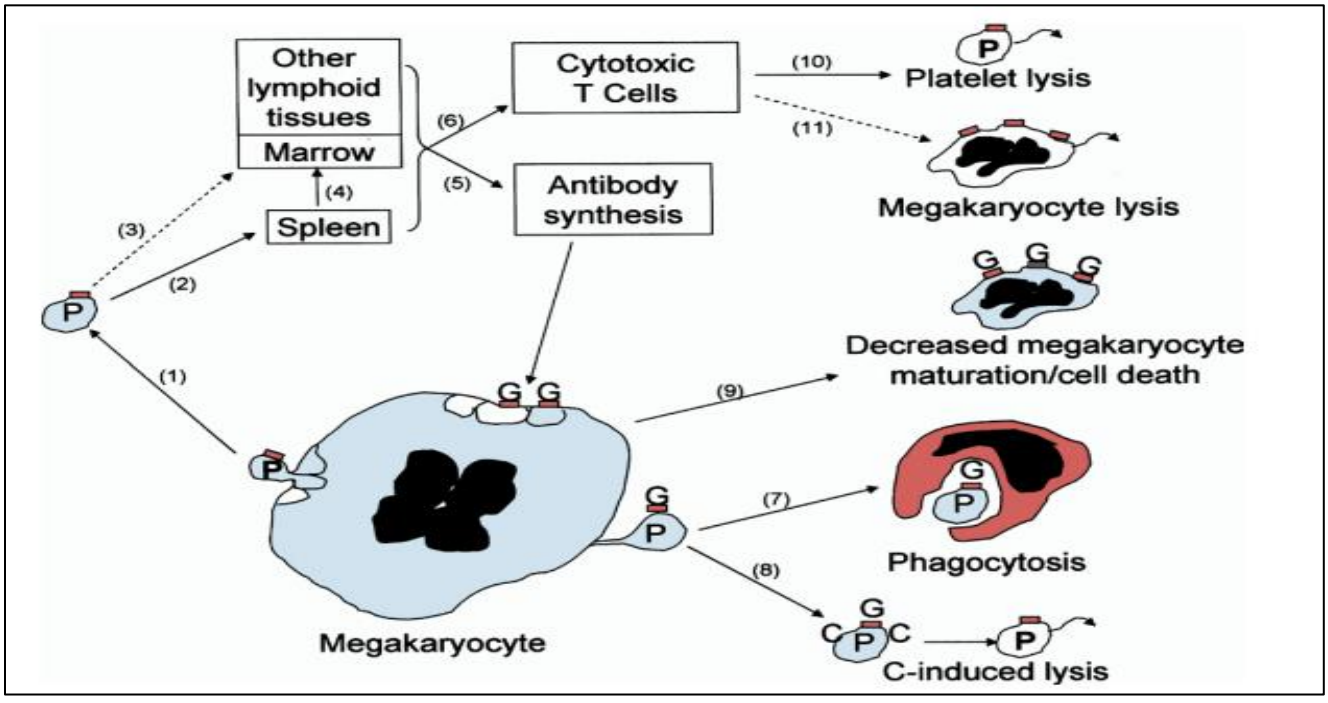
International Working Group (IWG) formerly classified the disease in two different phases based in the natural history of ITP. ITP was defined as ‘‘CHRONIC’’ if it lasted for more than 6 months and ‘‘ACUTE’’ if it was a self-limited and transient disease. The IWG members lately made a consensus upon the three distinct phases of the disease

- **Newly Diagnosed ITP** – up to three months, from the time of diagnosis
- **Persistent ITP**- disease duration ranges between 3 and 12 months from diagnosis
- **Chronic ITP**- classified for a disease duration of more than 12 months



PATHOPHYSIOLOGY OF ITP

- Platelet membrane proteins become antigenic and stimulate the immune system to produce autoantibodies and cytotoxic T cells
- Antigen-specific memory cells develop, circulate, and initiate a more generalized immune response”
- IgG antiplatelet antibody (G) is produced and cytotoxic T lymphocytes are generated
- Autoantibody binds to platelets, causing their destruction by either phagocytosis or complement (C)-induced lysis and to megakaryocytes resulting in decreased maturation and cell death
- Cytotoxic T lymphocytes result in platelet lysis and, possibly, megakaryocyte lysis
- Solid lines show reactions with solid experimental evidence and dashed lines show reactions that are postulated



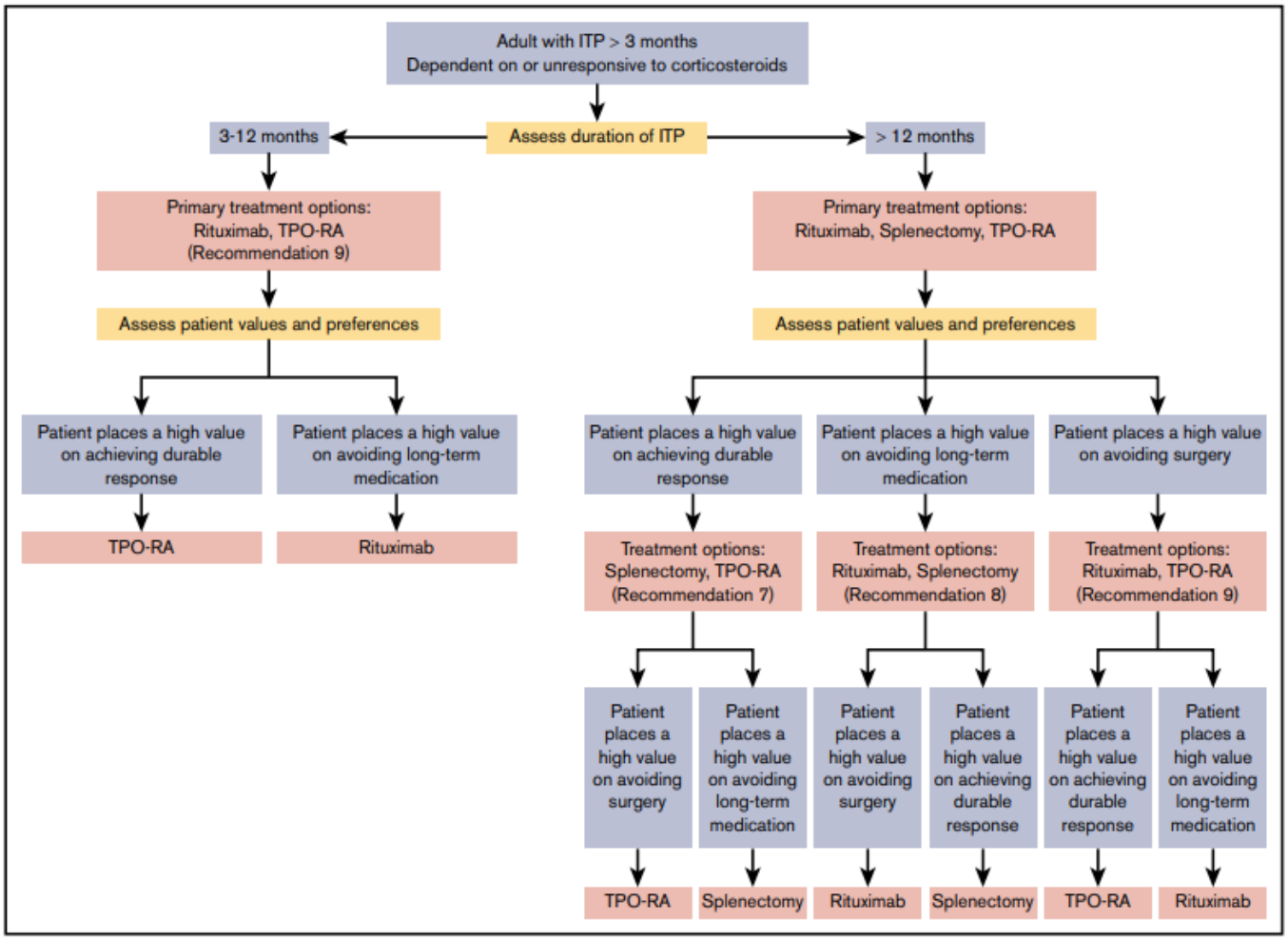
ITP - TREATMENT ALGORITHMS

Clinical Practice Guidelines for US

University of Oklahoma Health Sciences Center (OUHSC) published the treatment related guidelines for the management of Immune Thrombocytopenia (ITP).

The guideline review takes into consideration the available evidence and recommendations regarding first- and second-line management of adults and children with ITP.

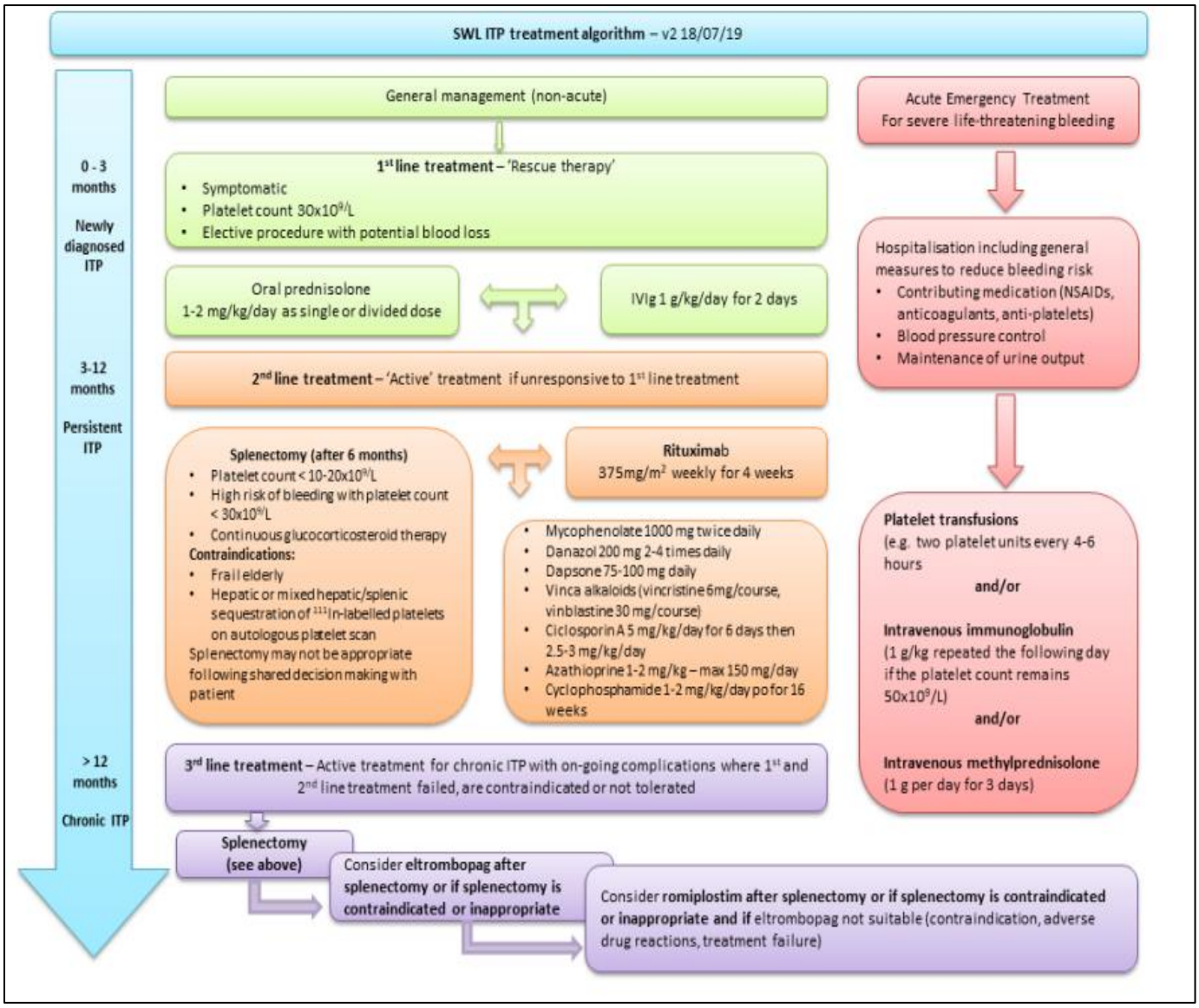
The selection of second-line therapy in adults with ITP is recommended to be individualized based on patient preferences and the time period of the disease.



Clinical Practice Guidelines for EU

According to the treatment guidelines for ITP in European Union individualized specific therapy for ITP may not be necessary unless the platelet count is < 10x109/L or there is extensive bleeding.

The patient's age, severity of the illness and anticipated natural history is taken into the consideration for the clinical management of this condition.



Clinical Practice Guidelines for Japan

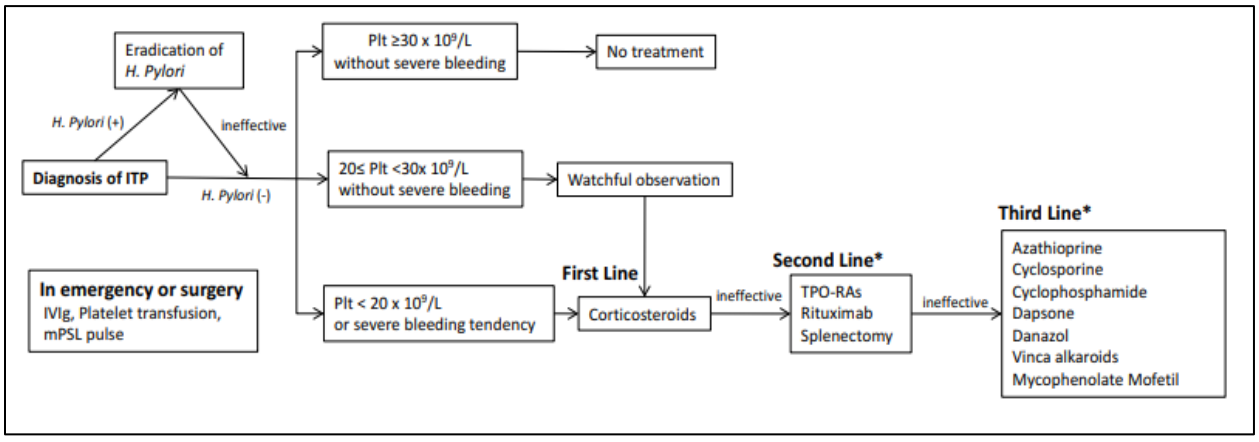
The clinical practice guidelines suggest the treatment is decided based on the bleeding symptoms and platelet count.

The first-line treatment is corticosteroids. Patients who do not achieve the therapeutic target with corticosteroids or require long-term administration of high-dose corticosteroids, or patients unable to tolerate corticosteroids due to complications or adverse drug reactions, are transitioned to second-line treatment."

TPO-RAs, rituximab and splenectomy are recommended as second-line treatments. Selection of the second-line treatment should be based on consideration of the advantages and disadvantages of each treatment, and to suit the situation of each individual patient.

Third line treatment should be considered for refractory ITP cases where the second-line treatment is ineffective or is difficult to implement due to complications or other factors, after fully considering the necessity of the treatment.

High-dose intravenous immunoglobulin, methylprednisolone pulse therapy, or platelet transfusion should be considered for patients with severe bleeding symptoms, marked thrombocytopenia or who urgently require an increase in their platelet count because of surgery or for some other reason.

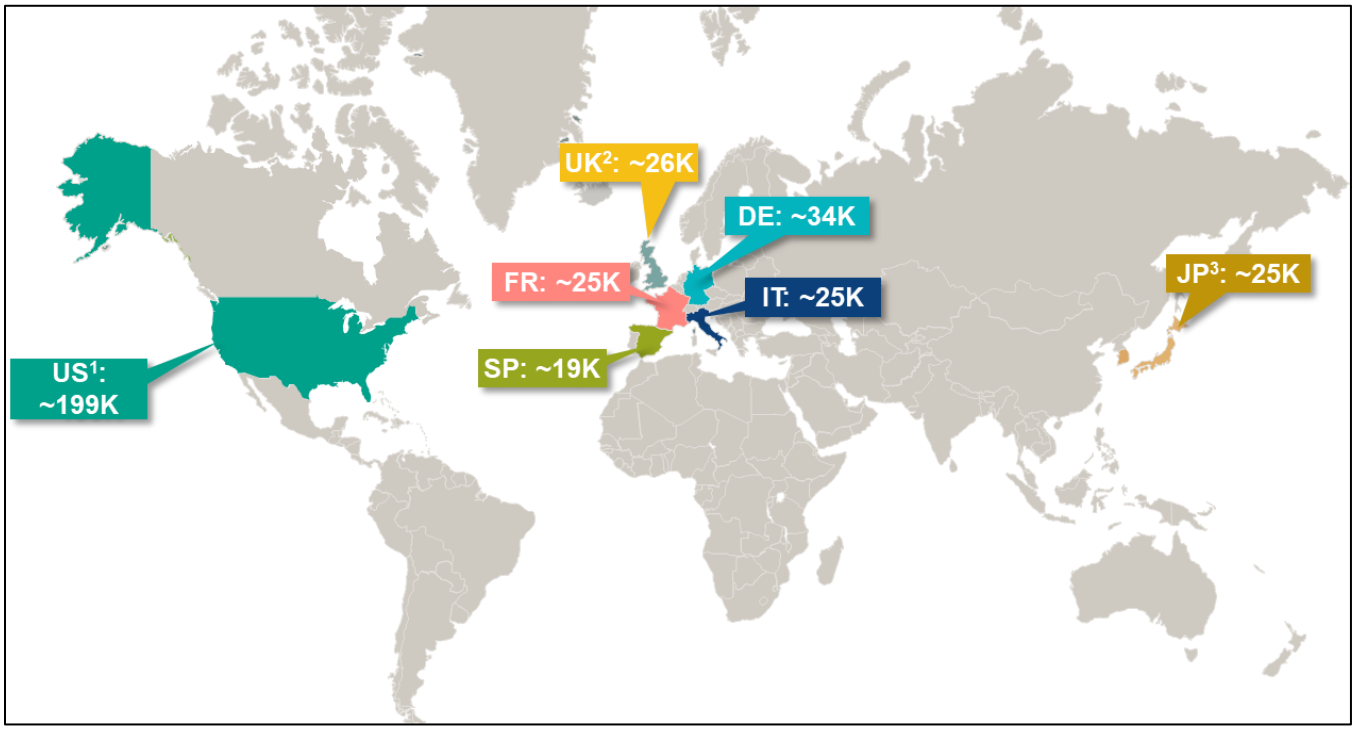


EPIDEMIOLOGY OF THE DISEASE

Prevalence Rates

Region	US[1]	UK[2]	Japan[3]
Title	Analysis of the impact and burden of illness of adult chronic ITP in the US.	Prevalence of Diagnosed Adult Immune Thrombocytopenia in the United Kingdom.	Reference guide for management of adult immune thrombocytopenia in Japan: 2019 Revision
Study period (Publication)	2000 to 2004 (2009)	1992 to 2009 (2011)	2019
Sample Size/ Methodology	Retrospective claims data	General practice research database	Estimation based on disease medical care certificates issued
Data Points	80 per 100,000	50 per 100,000	25000 patients
Prevalence (2020)	~199K	~26K	~25K
Authors	Saleh MN et.al.	Bennett, D. et.al.	H. Kashiwagi et al.

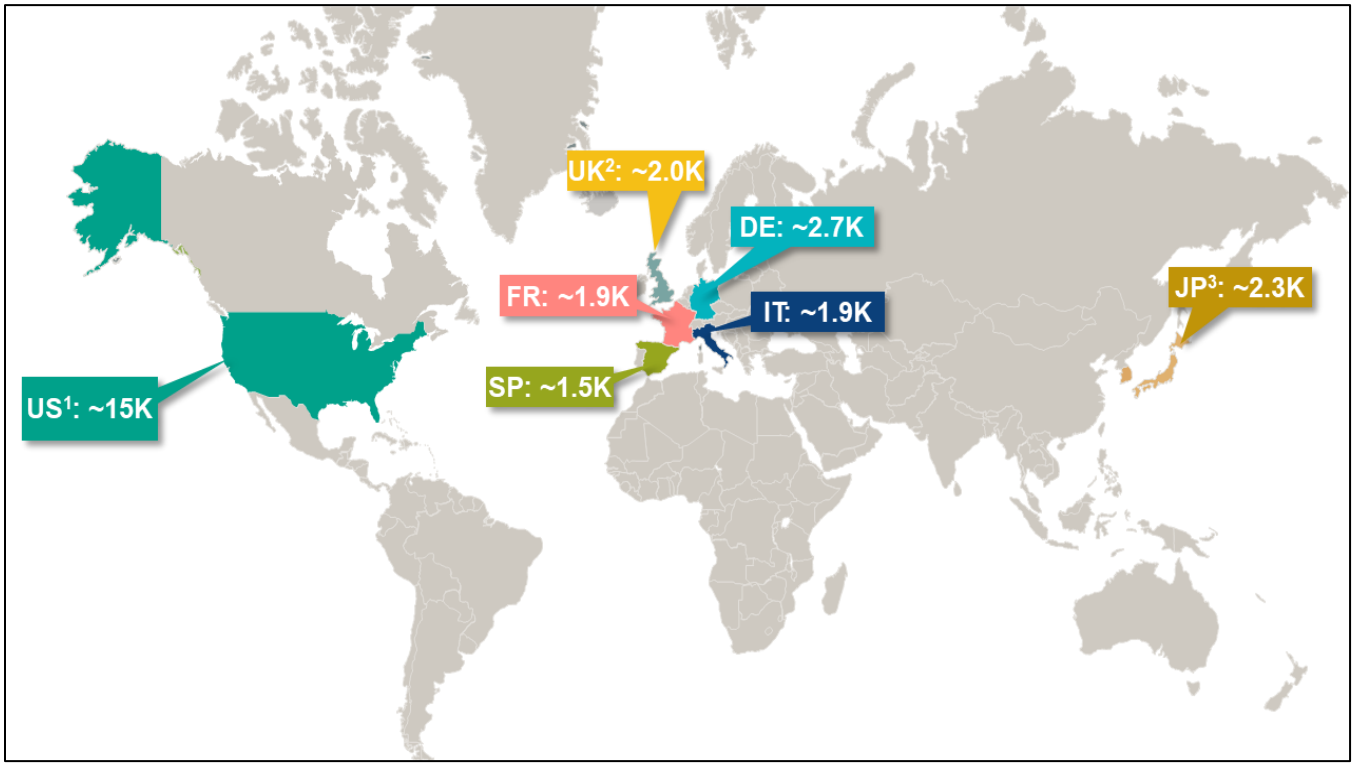
Prevalence Rate Depiction



Incidence Rates

Region	US[1]	UK[2]	Japan[3]
Title	Primary immune thrombocytopenia in us clinical practice: incidence and healthcare burden in first 12 months following diagnosis.	The incidence of idiopathic thrombocytopenic purpura among adults: a population-based study and literature review.	Epidemiology of primary immune thrombocytopenia in children and adults in Japan: a population-based study and literature review.
Study period (Publication)	2010 to 2016 (2020)	1992 to 2005 (2009)	2004 to 2007 (2011)
Sample Size/ Methodology	Longitudinal patients claims data analysis	General practice research databased	DHMC database of the MHLW, Japan
Data Points	6.0 per 100,000	3.9 per 100,000	2.2 per 100,000
Incidence (2020)	~15K	~2K	~2.3K
Authors	Weycker, D et.al.	Abrahamson PE et.al.	Y Kurata et.al.

Incidence Rate Depiction

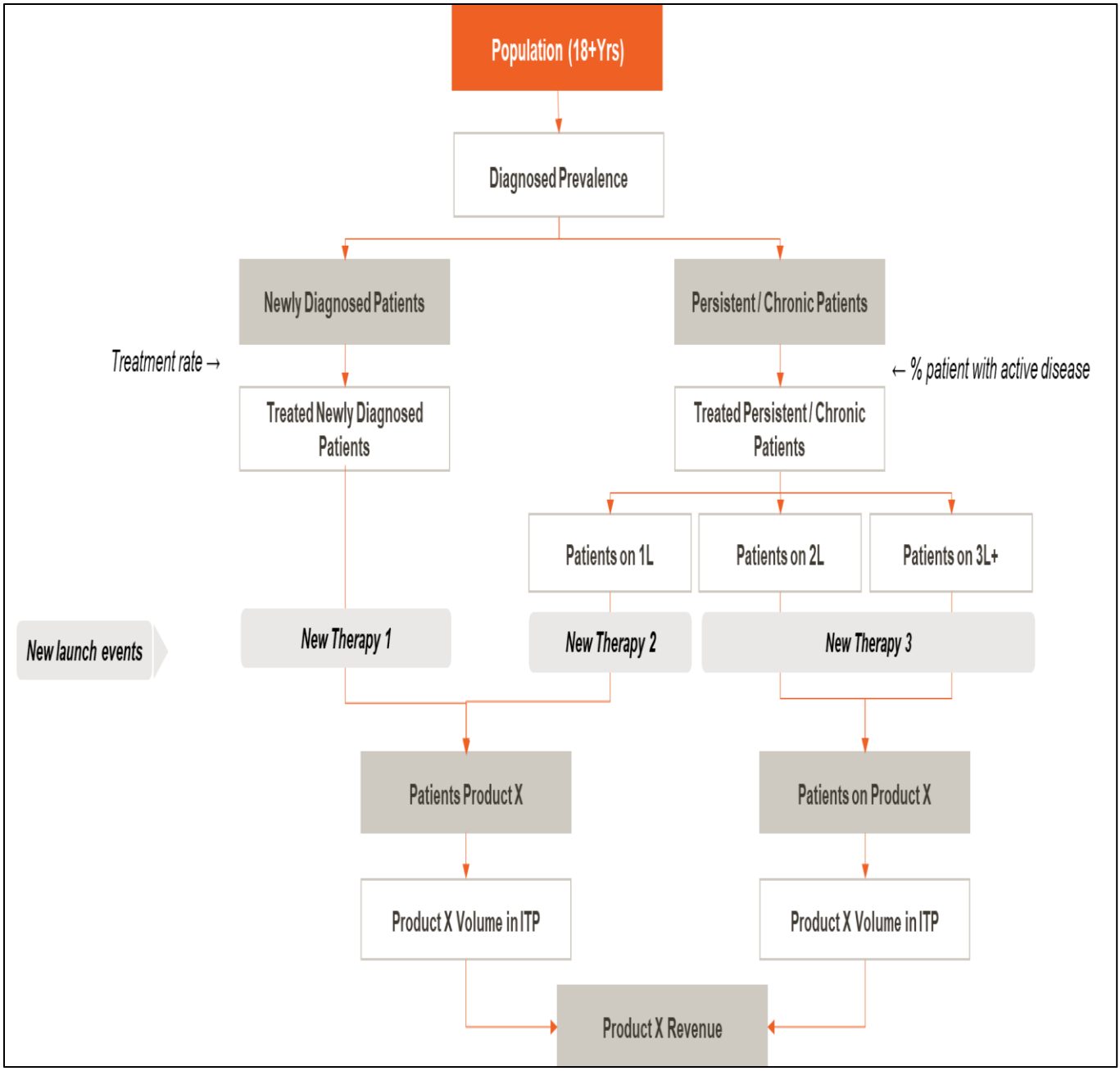


sFORECAST FLOW

Based on the treatment algorithm and epidemiological considerations a proposed forecast flow is generated.

The aim is to estimate the yearly revenue from product X by calculating the patient numbers and the volume consumption by each patient. A simple diagrammatic representation of the proposed forecast flow is depicted below.

The flow takes into consideration the yearly population of adults effected with ITP and classifying them into Newly Diagnosed patients and persistent + chronic patients. a treatment rate cut of the patients is applied to identify their patients with active disease. The patients are further classified into 1st line, 2nd Line and #rd line patients based on the disease characteristics. From there an estimated number is generated for the patients using the product X and the quantity each patient consumes on annual basis. The total quantity so calculated for each patient is then multiplied by the total treated patient numbers and the annual cost of therapy to achieve a final revenue share or the product.



Proposed Forecast Flow for ITP

RESULTS AND CONCLUSION

The study report discusses about the disease definition and Pathophysiology in detail. It can be noticed that there may not be a specific cause of the disease, the disease itself is an autoimmune disorder causing lack of platelets in the body.

The treatment guidelines vary from region to region, but no significant changes can be observed in the studied three treatment guidelines.

Several studies were also referred to collect the epidemiological data for the disorder. Data points for prevalence and incidence were ordered for each of the regions and the estimated patient numbers were so calculated by multiplying with the total population. The numbers reported are for the current year.

The data & assumptions acquired from secondary research were used to develop an excel based forecast model for ITP. The model was split by different lines of therapies to incorporate the patient dynamics across various therapies. Various market dynamics and assumptions were also taken into consideration for deciding the forecast revenue from the model.

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