



# Assessment of the hemophilia treatment market

*Case study*

**boston**ANALYTICS

*(A division of Pythhos Technology LLC)*

# BA developed a market assessment of the hemophilia market opportunity for a US-based technology consulting firm

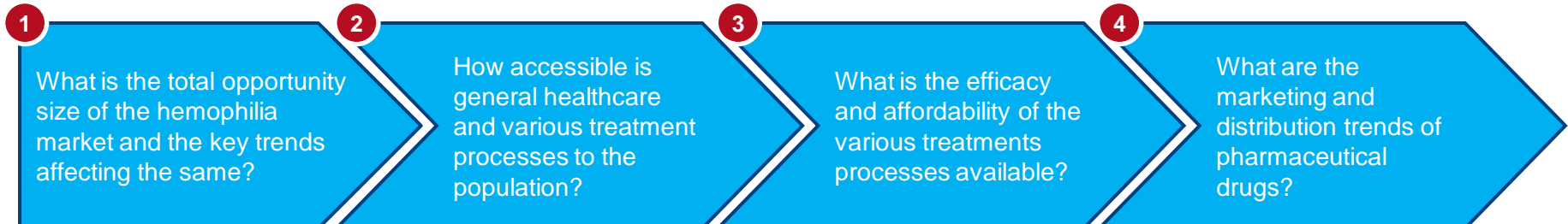
## Client Background

- The client is a U.S.-based strategy and technology consulting organization providing management, technology and services to primarily civilian government agencies, defense and intelligence agencies
- The client wanted to identify the existing opportunity and characteristics of the hemophilia markets in UAE, Saudi Arabia, South Africa, Egypt and India

## Key Highlights

- The market assessment study entailed identification of the hemophilia market opportunity focusing on:
  - Size of the hemophilia market based on numbers diagnosed and key trends affecting the same
  - Market access to the general healthcare and various treatment processes available
  - Efficacy and affordability of various treatment processes available
  - Distribution and marketing of pharmaceutical drugs in the selected countries
  - Country-specific stakeholders and their characteristics

## Key Business Questions



# The total opportunity size of the hemophilia market was developed along with a view of key trends

1

What is the total opportunity size of the hemophilia market and the key trends affecting the same?

2

How accessible is general healthcare and various treatment processes to the population?

3

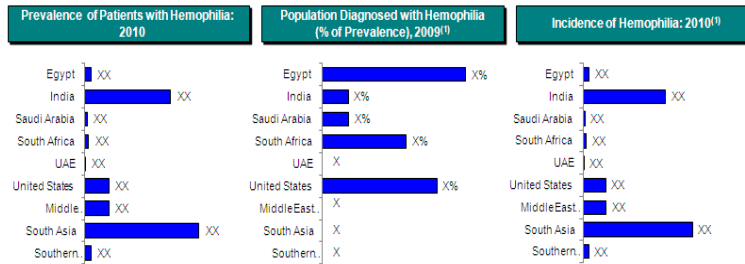
What is the efficacy and affordability of the various treatments processes available?

4

What are the marketing and distribution trends of the pharmaceutical drugs?

## The proportion of people diagnosed with hemophilia is relatively high in Egypt and South Africa among the selected countries

Illustrative



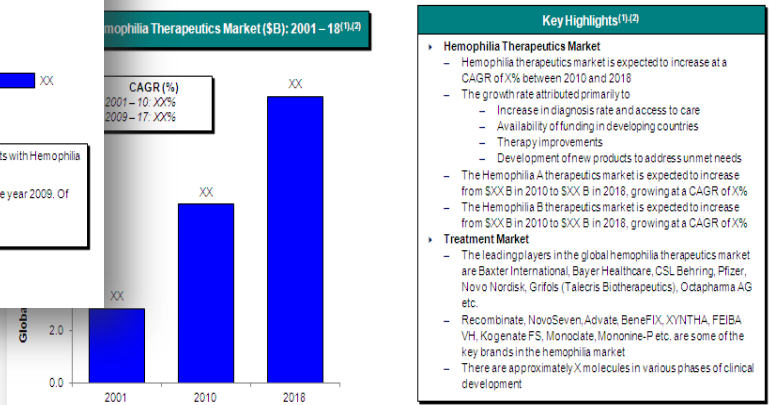
- Globally, prevalence of hemophilia patients is approximately XX, of these XX(i.e. XX%) patients with Hemophilia A and XX(i.e. XX%) patients with Hemophilia B
- According to report published by World Federation of Haemophilia, total number of people diagnosed with Hemophilia A and B was XX in the year 2009. Of which number of people diagnosed with Hemophilia A was XX and Hemophilia B was XX
- XX% of people around the world do not have access to treatment, according to National Hemophilia Foundation, USA

Source:  
 (1) Report on the Annual Global Survey 2009, World Federation of Hemophilia, <http://www.wfhh.org/publications/files/pdf/092.pdf>  
 (2) [Baylor Science Center, Baylor Medical Center, Baylor Hemophilia Center](http://www.baylor-science.org/med/medschool/department_of_pediatrics/hemophilia_center/)  
 (3) [Statistics by Country for Hemophilia](http://www.hemophilia.org/what-is-hemophilia/about-hemophilia/)

BA also identified the key trends affecting the hemophilia market in the selected markets.

## Global Hemophilia therapeutics market is expected to be XX

Illustrative



- ### Key Highlights<sup>(1)(2)</sup>
- Hemophilia Therapeutics Market**
    - The Hemophilia therapeutics market is expected to increase at a CAGR of XX% between 2010 and 2018
    - The growth rate attributed primarily to
      - Increase in diagnosis rate and access to care
      - Availability of funding in developing countries
      - Therapy improvements
      - Development of new products to address unmet needs
    - The Hemophilia A therapeutics market is expected to increase from \$XX B in 2010 to \$XX B in 2018, growing at a CAGR of XX%
    - The Hemophilia B therapeutics market is expected to increase from \$XX B in 2010 to \$XX B in 2018, growing at a CAGR of XX%
  - Treatment Market**
    - The leading players in the global hemophilia therapeutics market are Baxter International, Bayer Healthcare, CSL Behring, Pfizer, Novo Nordisk, Grifols (Talecris Biotherapeutics), Octapharma AG etc.
    - Recombinate, NovoSeven, Advate, BeneFIX, XYNTHA, FEIBA VH, Kogenate FS, Monoclate, Mononine-P etc. are some of the key brands in the hemophilia market
    - There are approximately X molecules in various phases of clinical development

Source:  
 (1) Hemophilia Therapeutics - Pipeline Assessment and Market Forecasts to 2018, <http://www.globenewswire.com/press-release/2016/05/10/544443/0/en/Hemophilia-Therapeutics-Pipeline-Assessment-and-Market-Forecasts-to-2018-report.html>  
 (2) 2017 Data Research Report on Hemophilia Market, <http://www.iihsresearch.com/Product-Details.aspx?ProductID=100000000>  
 (3) Hemophilia - Drug Pipeline Analysis and Market Forecasts to 2018, <http://www.globenewswire.com/press-release/2016/05/10/544443/0/en/Hemophilia-Drug-Pipeline-Analysis-and-Market-Forecasts-to-2018-report.html>

# Accessibility of general healthcare and other treatment processes in the selected markets were determined

1

What is the total opportunity size of the hemophilia market and the key trends affecting the same?

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How accessible is general healthcare and various treatment processes to the population?

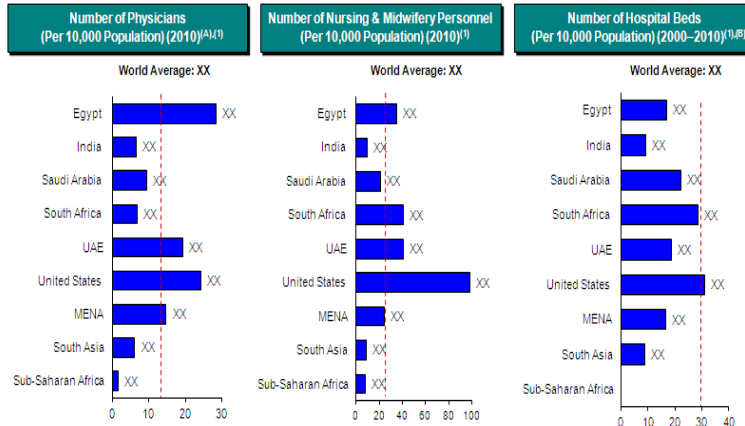
3

What is the efficacy and affordability of the various treatments processes available?

4

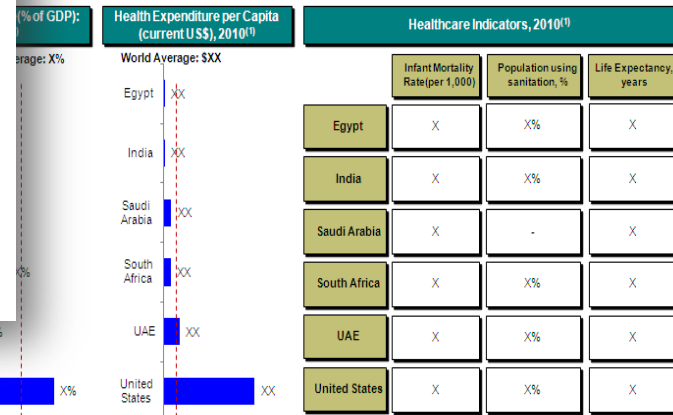
What are the marketing and distribution trends of the pharmaceutical drugs?

## South Africa and United Arab Emirates has better health resources than most of the other countries Illustrative



Note:  
 (A) Data for South Africa is for the year 2001.  
 (B) The range indicates the latest available figures within the time period 2000-2010.  
 Source:  
 (1) World Health Statistics 2010, WHO: [http://www.who.int/whosis/whosis/EN/10/EN10\\_Full.pdf](http://www.who.int/whosis/whosis/EN/10/EN10_Full.pdf)

## Health expenditure as percentage of GDP is below world average in all the selected countries; ranges between 3.7% to 8.9% Illustrative



Note:  
 (1) Global Immunization measures the percentage of children ages 12-23 months who received acellular pertussis before 12 months or early time spanned diphtheria, pertussis or whooping cough, and tetanus (DPT).  
 Source:  
 (1) World Bank Data: <http://data.worldbank.org>, accessed on 07 December 2012.

# Efficacy and affordability of treatment processes available in the market were evaluated

1

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## Factor concentrates (FVIII and FIX) are the most effective choice for haemophilic A and B patients respectively Illustrative

List of hemophilia Treatment Methods		UAE	Saudi Arabia	South Africa	Egypt	India	US
Clotting Factor Concentrates	<b>Viral-inactivated plasma-derived or recombinant FVIII concentrate</b> <ul style="list-style-type: none"> <li>FVIII is the drug choice for treatment of haemophilia A</li> <li>There are two available types of factor VIII concentrates i.e.                             <ul style="list-style-type: none"> <li>Recombinant factor VIII concentrates (rFVIII)</li> <li>Plasma-derived factor VIII concentrates (pdFVIII)</li> </ul> </li> </ul>	✓	✓	✓			
	<b>Viral-inactivated plasma-derived or recombinant FIX concentrate</b> <ul style="list-style-type: none"> <li>FVIII is the drug choice for treatment of haemophilia B</li> <li>There are 2 available types of factor VIII concentrates i.e.                             <ul style="list-style-type: none"> <li>Recombinant factor IX concentrates (rFIX)</li> <li>Plasma-derived factor IX concentrates (pdFIX)</li> </ul> </li> </ul>		✓	✓			
	<b>Other Plasma Products</b> <ul style="list-style-type: none"> <li>These drugs include the non-viral-inactivated plasma products such as cryoprecipitate or fresh frozen plasma (FFP) products</li> <li>Cryoprecipitate is preferable to FFP for the treatment of haemophilia. FFP is not recommended due to safety issues</li> <li>These products can be used to treat haemophilia B in countries that are unable to afford plasma-derived FIX concentrates</li> </ul>				✓		
	<b>Desmopressin acetate</b> <ul style="list-style-type: none"> <li>Desmopressin (also known as DDAVP) is a synthetic hormone that stimulates the release of factor VIII</li> <li>It is a drug of choice primarily for patients with mild haemophilia A who have minor bleeding</li> </ul>		✓	✓			
<b>Prophylactic Therapy</b> <ul style="list-style-type: none"> <li>Prophylaxis is the treatment by intravenous injection of factor concentrates in order to prevent anticipated bleeding</li> <li>The treatment is of two types:                             <ul style="list-style-type: none"> <li>Episodic (on-demand) treatment which is given at the time of clinically evident bleeding</li> <li>Continuous treatment which is initiated either before or after the onset of osteoarthral/joint disease</li> </ul> </li> </ul>	✓	✓	✓				

✓ Denotes the most commonly used treatment methods in the select countries

## Certain adjunctive and home treatment methods may be a Illustrative addition to the factor replacement therapy to manage/prevent bleeding

List of hemophilia Treatment Methods		UAE	Saudi Arabia	South Africa	Egypt	India	US
Adjunctive Management Therapy	<b>First-Aid Measures</b> <ul style="list-style-type: none"> <li>This is an adjunctive therapy in addition to increasing factor level with clotting factor concentrates</li> <li>These measures include protection (splint), rest, ice, compression, and elevation (PRICE) which may be used for bleeding in muscles and joints</li> </ul>					✓	
	<b>Physiotherapy/ Rehabilitation</b> <ul style="list-style-type: none"> <li>Physiotherapy/rehabilitation is particularly important for functional improvement and recovery in patients with musculoskeletal bleeds and for those with established hemophilic arthropathy</li> <li>This treatment must be combined with the primary treatment methods such as prophylactic treatment to be effective</li> </ul>			✓		✓	
	<b>Antifibrinolytic drugs</b> <ul style="list-style-type: none"> <li>Antifibrinolytic drugs such as tranexamic acid or epsilon aminocaproic acid) are effective for mucosal bleeds and dental extractions</li> <li>Regular treatment with only these products is of no value in the prevention of hemarthroses in haemophilia</li> </ul>		✓	✓			✓
	<b>Home Therapy</b> <ul style="list-style-type: none"> <li>Haemophilic patients can be treated at a home setting wherever appropriate</li> <li>This allows immediate access to clotting factor and hence optimal early treatment, resulting in decreased pain, dysfunction, and longterm disability</li> <li>Surgery may be required for patients with haemophilia-related complications or unrelated diseases</li> <li>Surgery should be preferred only after consultation with a comprehensive hemophilia treatment center</li> </ul>		✓			✓	✓
<b>Pain Management</b> <ul style="list-style-type: none"> <li>Certain basic treatment processes might be used for pain management in patients suffering from pains due to chronic hemophilic arthropathy, joint or muscle bleeding. Few examples include COX-2 inhibitor, paracetamol, morphine etc</li> </ul>						✓	

✓ Denotes the most commonly used treatment methods in the select countries

# BA studied the marketing and distribution trends of drugs in the markets to develop a comparative picture across markets

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What are the marketing and distribution trends of the pharmaceutical drugs?

## While medical detailing is allowed in Egypt, prior permission is required for advertisement **Illustrative**

	Allowed: Yes / No	Guidelines	Common Practices
<b>Medical Detailing</b>	Allowed	Legal for doctors to accept anything in cash or kind from pharmaceutical companies	Companies influence physicians through elements such as detailing, drug sales journal advertising, conferences and research grants and sponsoring of education programs Medical representatives along with elements significantly influence pharmaceutical advertising, expensive branded
<b>Advertising</b>	Prior permission required	Prior approval of the technical committee for drug control is mandatory for advertising pharmaceutical products	Direct-to-consumer advertising (DTC) and promotion are among the widely used Journal advertising, direct mail, etc. are not widely used within the Egyptian industry

Sources:  
 (1) <http://www.fairpharm.com/forum/showthread.php?p=43264>  
 (2) <http://ome.yale.edu/ris-proceedings/P33%20Papers/Sama%20Vehia.pdf>

## Medical detailing is allowed both in Saudi Arabia and South Africa, prior permission is not required for advertisement in South Africa **Illustrative**

	Allowed: Yes / No	Guidelines	Common Practices
<b>Medical Detailing</b>	Allowed	Not available	Not available
<b>Advertising</b>	Not available	Saudi Food & Drug Authority is the regulatory authority in Saudi Arabia	Not available

	Allowed: Yes / No	Guidelines	Common Practices
<b>Medical Detailing</b>	Allowed	All medicines sold in South Africa must be registered by the Medicines Control Council (MCC)	No trend of doctors being bribed / given gifts by pharmaceutical companies is observed in South Africa
<b>Advertising</b>	Allowed	All advertising must be based on the approved claims for the medicine Advertising does not require prior approval by the MCC The MCC Inspectorate deals with any infringement as a contravention of the regulations	Not available

Sources:  
 (1) <http://www.sfd.gov.sa/En/Drug/Tools/Regulations---Guidelines.htm>

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