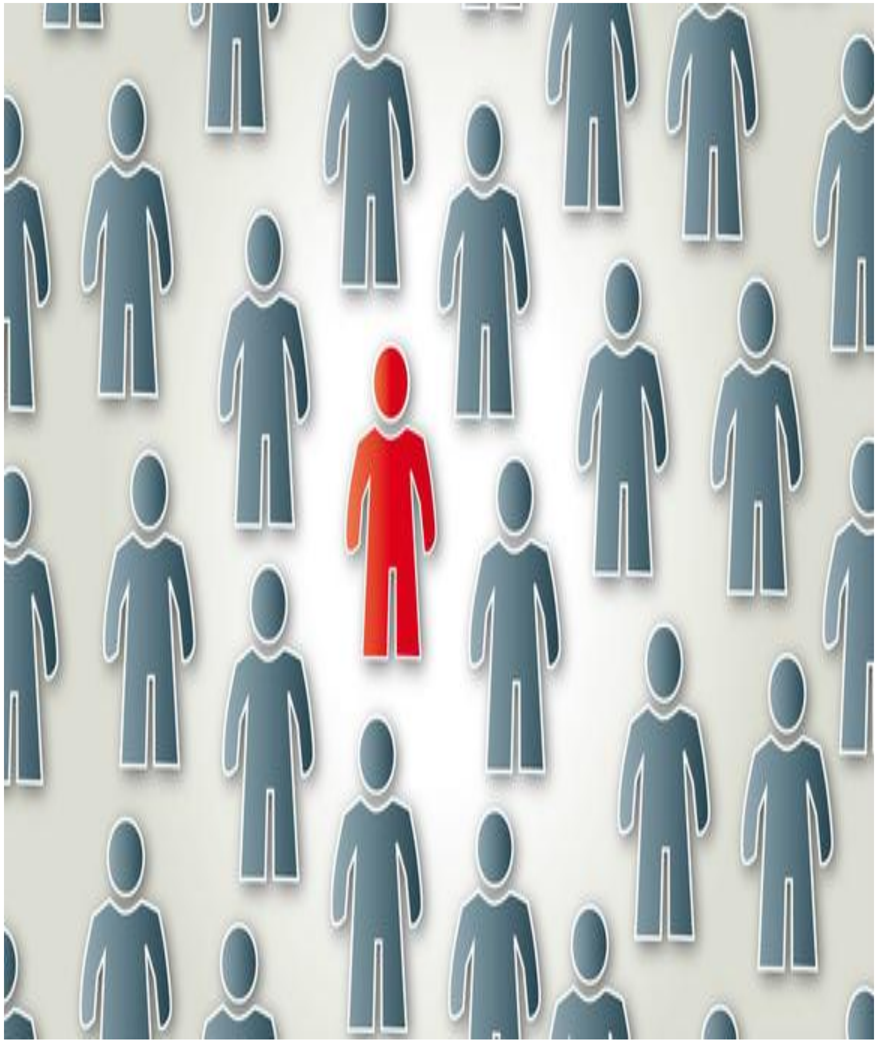
Market Oppurtunity assesment for Orphan / Rare diseases



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Organization Profile

- ZS Associates is one of the world's largest consulting firm focused exclusively on improving business performance through sales and marketing solutions, from customer insights and strategy to analytics, operations and technology.

- More than 3,000 ZS professionals in 21 offices worldwide draw on deep industry and domain expertise to deliver impact where it matters for clients across multiple industries, primarily focussing on Pharmaceutical and Biotech Industries.

- ZS focuses exclusively on the two areas that create customer demand: sales and marketing. As a result, the expertise in this domain is both broad and deep.

- They provide a vast range of marketing- and sales-related services in the industry, from customer insight, product development, and sales and marketing strategies, to sales compensation planning and even plan administration, to name just a few.

Activities done during Dissertation

Therapy Area Overviews

- Created therapy area overview for three Orphan indications :
- Fabry Disease
- Hereditary Angioedema
- Paroxymal Nocturnal Haemoglubinuria

Study of Orphan diseases and Products

- In response to the requirement and need raised by pharma clients, the study involved the identification of Orphan indication present currently taking specific parameters into consideration. Thereafter,listing down all the Orphan designated products currently in the market and in pipeline.

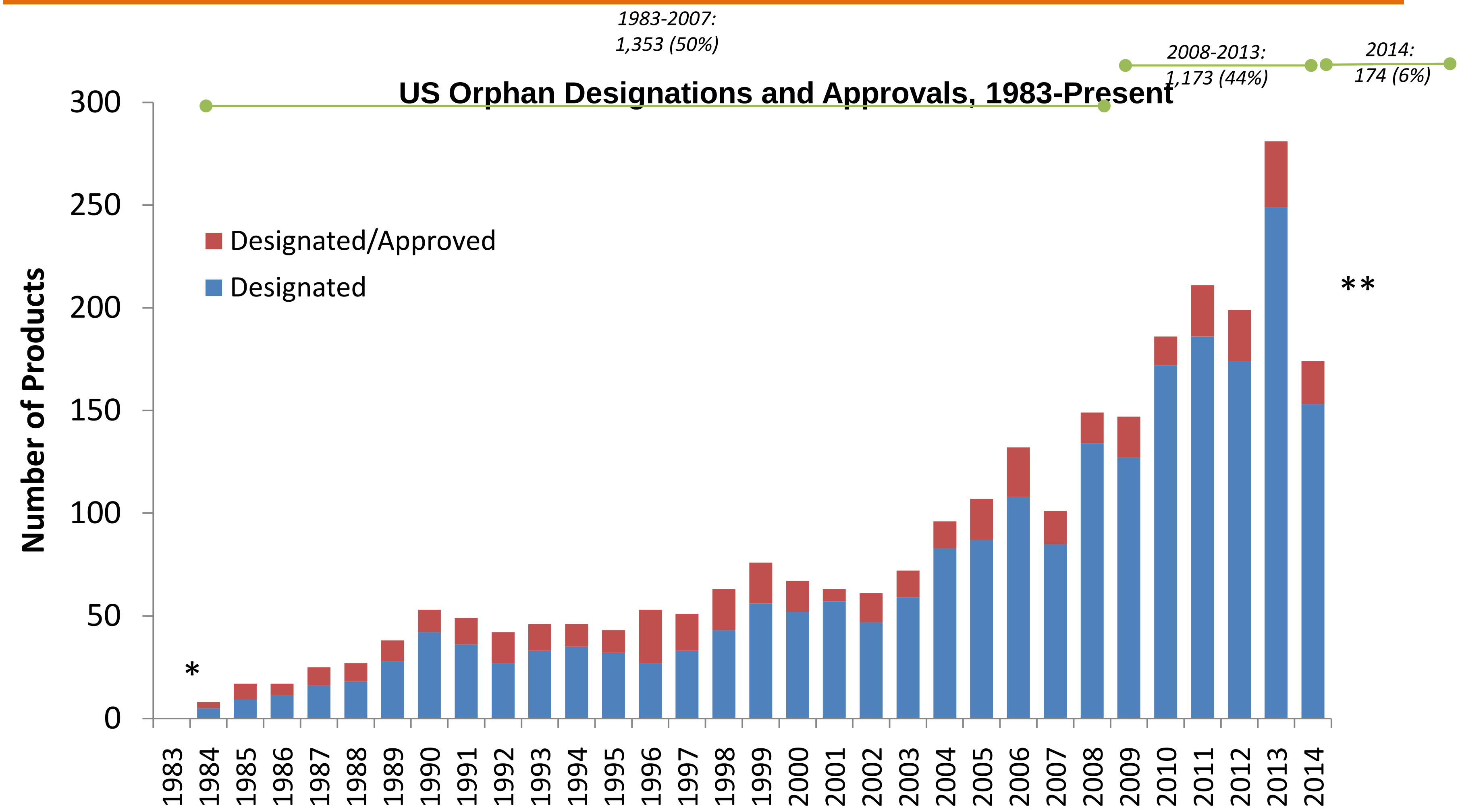
Data collection for PMSA Conference

- Created a Unique list of top EMR data vendors and ranked them on three parameters :
- Physician count
- Patient Coverage
- Hospital / Clinics covered.

Orphan Indications and Orphan Drugs

- **Orphan diseases** are defined as indications affecting fewer in thousand patients.They are also called “Rare diseases”.
- In the USA it is defined as one that affects fewer than 200 000 individuals, but in Japan the number is 50 000 and in Australia 2000 . These numbers clearly relate to the population sizes of these countries.
- At present , there are almost 7,000 Orphan indications present in number.
- **Orphan drugs** are medicinal products intended for diagnosis, prevention or treatment of life-threatening or debilitating rare diseases.
- They are "orphans" because the pharmaceutical industry has little interest under normal market conditions in developing and marketing drugs intended for only a small number of patients suffering from very rare conditions
- While Orphan drugs target much Smaller populations then traditional mainstream drugs , the high cost of therapy and various developmental drivers , makes them an attractive investment for Pharmceutical companies.

US Orphan designations and Approval trends



Many small pharma companies have placed a significant amount of focus on the orphan and ultra orphan space

SMALL SIZED PHARMA

Shire

- Has been competing in the orphan space since acquiring TKT in 2006
  - 4 marketed products (Elaprase, Firazyr, Replagal, Vpriv)
  - 3 products in clinical trials
  - 5 products in preclinical development

BIOMARIN

- Launched in 1997 to develop enzyme therapies to treat orphan diseases
  - 4 marketed products (Aldurazyme [with Genzyme], Naglazyme, Kuvan, Furdapase)
  - 7 programs in clinical trials

ALEXION

- Launched Soliris in 2007
  - Targeting additional Soliris development to 4 additional indications
  - 4 products in early clinical development

Amicus

- Founded in 2002 and has initiated 3 development programs in orphan / rare diseases

ACTELION

- Founded in 1997 to focus on conditions involving the endothelium
  - 3 marketed products in PAH
  - 1 marketed product in Gaucher
  - At least 6 development programs in orphan diseases

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Big pharma companies recognize the strategic value of the space and have explicit investment strategies

MEDIUM TO LARGE SIZED PHARMA

gsk

- Announced rare disease unit in 2010, building off of their Promacta business in the US, alliances with Prosensia and JCR pharma, and including Duchenne, Hunter, Fabry, Gaucher

NOVARTIS

- Markets 13 orphan drugs and has an additional 23 orphan designations within its portfolio
- Explicit strategy to get products to market quickly has led to multiple orphan approvals while the firm continues to pursue indications with larger patient populations

genzyme A SANOFI COMPANY

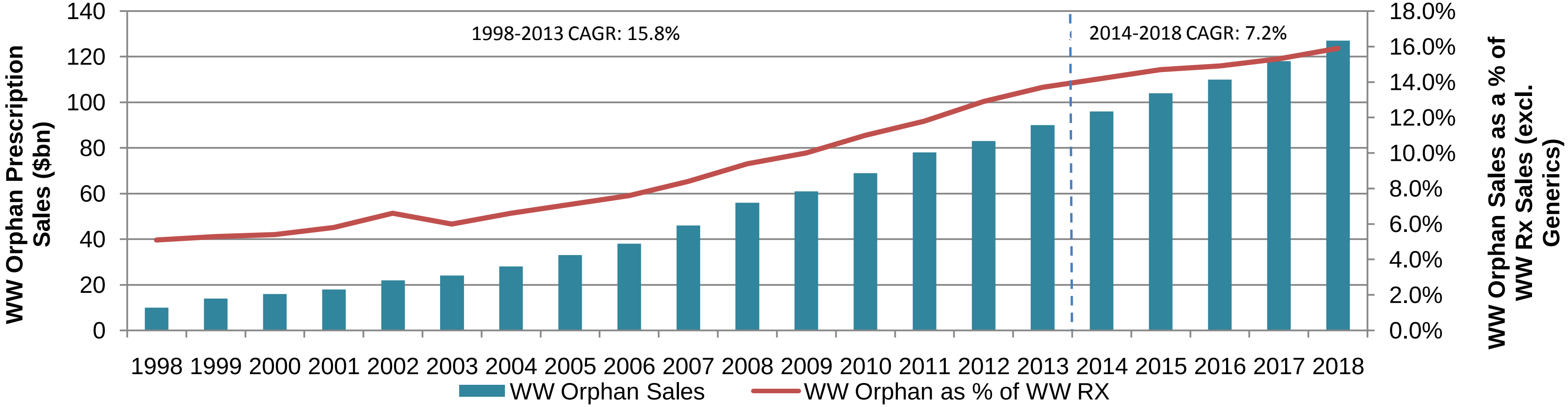
- Acquired Genzyme in 2011, with 6 commercial products in orphan and rare diseases, including Gaucher, Fabry and Pompe

Pfizer

- Acquired FoldRx and created orphan disease unit in 2010
- Assets under development in CF, Gaucher, Huntington and others
- Launched Xalkori in 2011, targeting <7% of non-small cell lung cancer patients

The worldwide orphan drug market is set to grow to $127bn , a CAGR of 7.2% per year between 2012 and 2018

Worldwide Orphan Drug Sales & Share of Prescription Drug Market (1998-18)



Market Size and Growth Estimates

- ~50% of the market is in the US, with the total market for orphan oncology drugs exceeding USD ~45 billion in 2013
- Historic growth has been ~25% annually and higher than non-orphan sales; orphan sales CAGR is expected to be larger than non-orphan for 2010 – 2030[1]
- Orphan market growth is expected to level out at 7.2% annually for the next 5 years, due to exclusivity expiring for current products outpacing new orphan products coming to market; regardless this still outpaces non-orphan market growth at 3.7%

Source: EvaluatePharma; Datamonitor; BCC Research; team analysis

The orphan drug space represents a large and growing opportunity for pharma & biotech companies, due to a variety of favorable dynamics

R&D Drivers

- R&D Grants
- Waived FDA Fees
- Shorter Development Timelines
- Tax Credits
- Greater Regulatory Success

Commercial Drivers

- Favorable Reimbursement
- Longer Exclusivity
- Lower Marketing Costs
- Fewer Hurdles to Approval
- Faster Uptake
- Premium Pricing

Rationale

Study Objectives

➢ To Understand the Market scenario (Global and US Specific) of Orphan drugs by identifying the number of Orphan indications present worldwide, the number of Pharmaceutical companies investing into Orphan space by taking the number of drugs marketed / pipeline for Orphan indications into consideration.

The Specific objectives were :

- To evaluate the Orphan flagged Pharmaceutical companies and the amount of work done by these companies in the Orphan marketspace.

- To analyze the drivers and challenges faced by the pharma companies for investing into this market.

Research Question

What is the current scenario of the Orphan (Global and US) market ?

Which are the companies currently investing in the Orphan drug space ?

What is the amount of work done by the identified companies in the Orphan Market in different practice areas and types?

Study Design

- **Type of study**: Exploratory Secondary Research
- **Geographies under study**:- United States and Global
- **Study subjects**: Orphan/Ultra-orphan indications and products
- **Data (information) to be collected**: Recording of Market scenario in study geographies under consideration.
- **Data collection tools**: ZS Specific data sources and publically available secondary research data sources was deployed to derive data

Methodology details

Study Design

- Exploratory Secondary research to identify the number of Orphan indications and the number of Pharmaceutical companies dealing in the Orphan Market space.

- Period of Study : Feb 2015 to May 2015

- On identifying the number of Orphan flagged Pharma Companies (N = 453) , detailed profiling of each company was done by recognizing the number of Orphan drugs they have in their Product portfolio.

- For distinguishing it more efficiently , Categorization of the Active Companies was done into segments based on the number of drugs they have in market and in development.

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Study Design

Identification of the Orphan Indications and Orphan flagged Pharmaceutical Companies

- Detailed Profiling of the indications
  - Number of drugs in the global Market,overall sales for that indication
  - Epidemiological data
    Incidence and Prevalance
- Creating a unique list of Orphan Active Pharma / biotech companies
  - Segmenting them on the basis of the number of Orphan drugs in their repertoire
  - Recognizing the High priority companies investing the most

Segmentation of the Active Companies into four divisions

Categories	Range
High pipeline / High Marketed	3-5 Orphan drugs in Pipeline 5-9 Orphan drugs Marketed
Low Pipeline / High Marketed	0-2 Orphan drugs in Pipeline 5-9 Orphan drugs Marketed
Low Marketed / High Pipeline	0-4 Orphan drugs in Market 3-5 Orphan drugs in Pipeline
Low Marketed / Low Pipeline	0-4 Orphan drugs in Market 0-2 Orphan drugs in Pipeline

Companies falling under High pipeline / High Marketed Segment were taken into priority only

Tabs Created to identify the number of Orphan drugs marketed and in development for the Active Companies

Company Name	Categories	Phase I	Phase II	Phase III	Total Pipeline	Total Marketed

Tabs Created to identify the number of Orphan flagged projects and further distinguishing them into Business area i.e Sales and Marketing

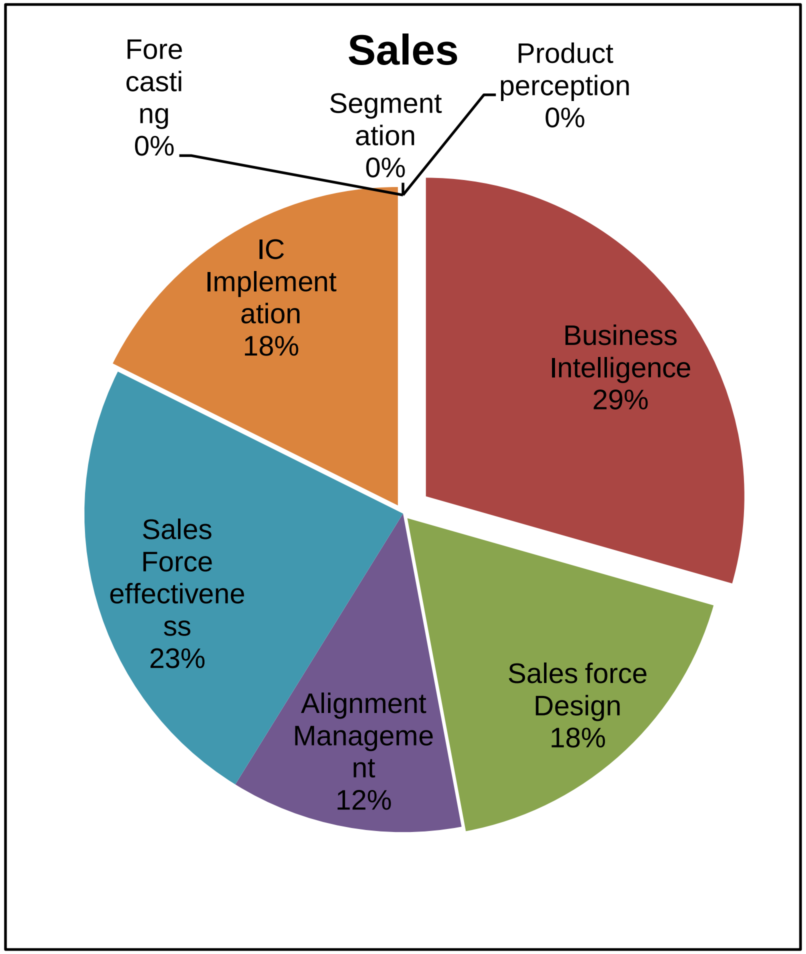
Total Orphan flagged projects	Sales	Marketing

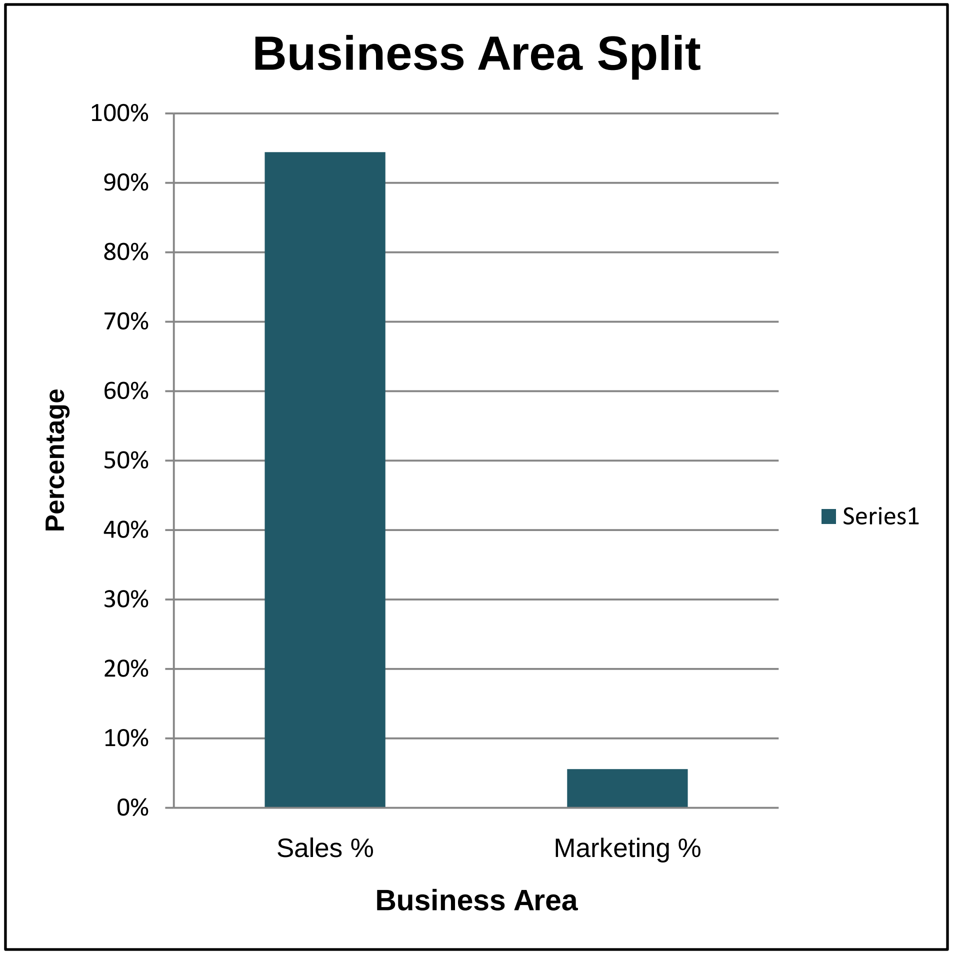
Tabs Created to identify the percentage of work done in different practice areas of each Business type i.e Sales and Marketing

Product Perception	Business Intelligence and Information	Sales force design	Alignment Managemen t	Sales Force effectivenes s	Forecasting	Segmentation	Pipeline Strategy

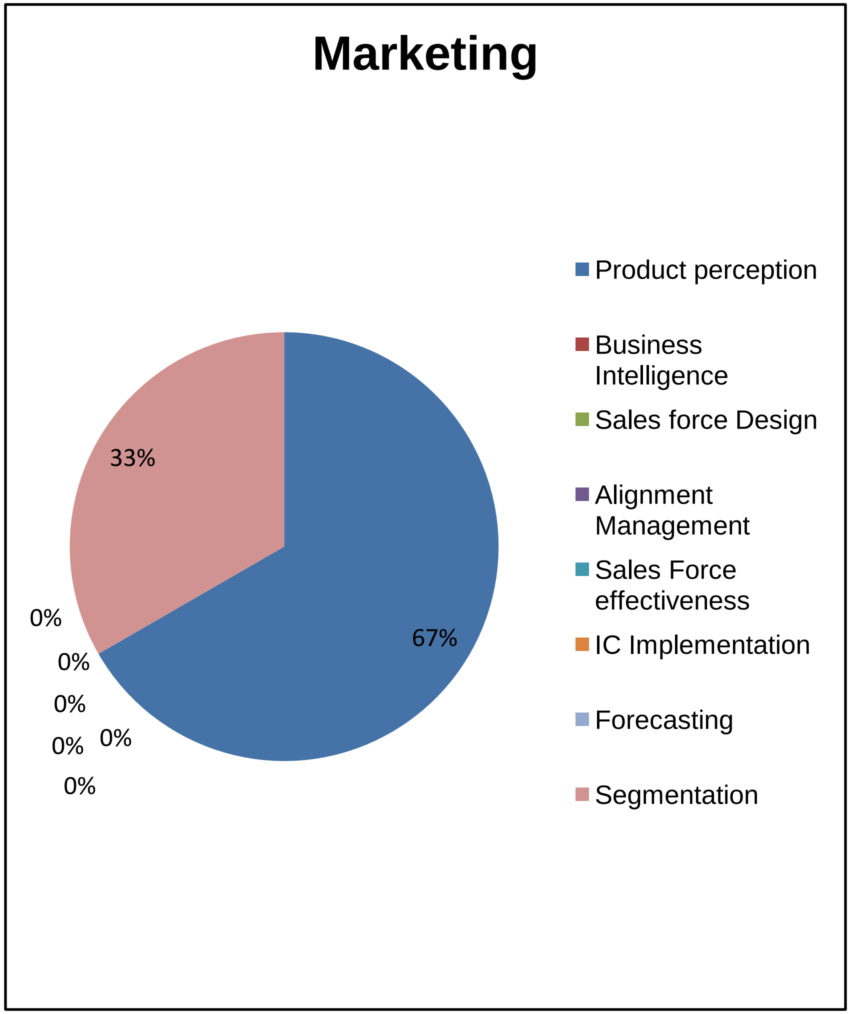
Findings

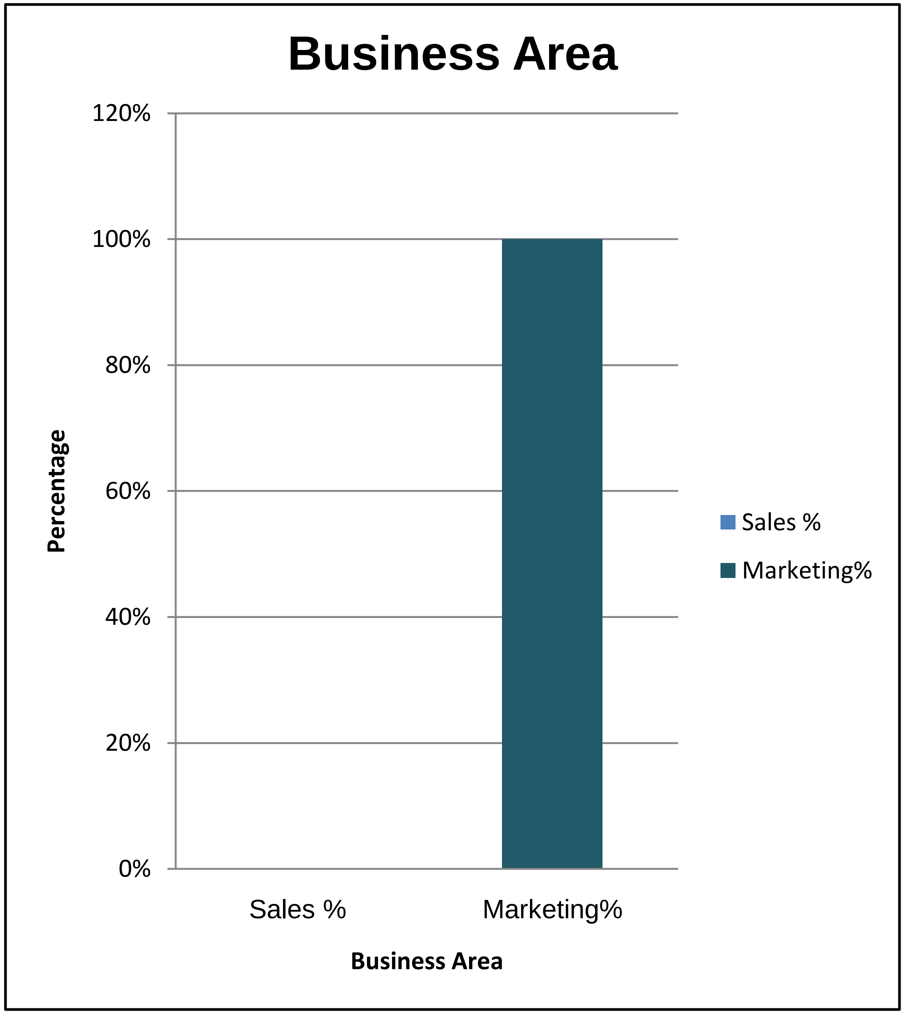
Shire Pharmaceuticals with 4 Orphan drugs in pipeline and 5 Marketed Globally was identified as a High priority Company and therefore was placed in the High Pipeline / High Marketed Segment



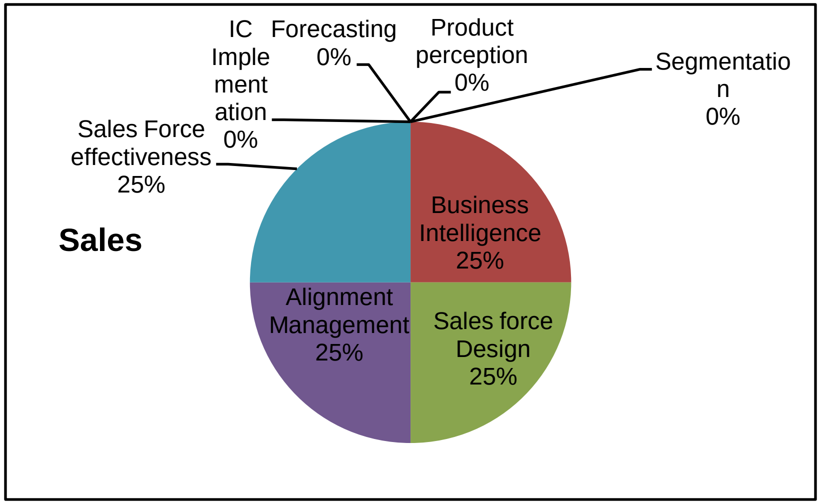


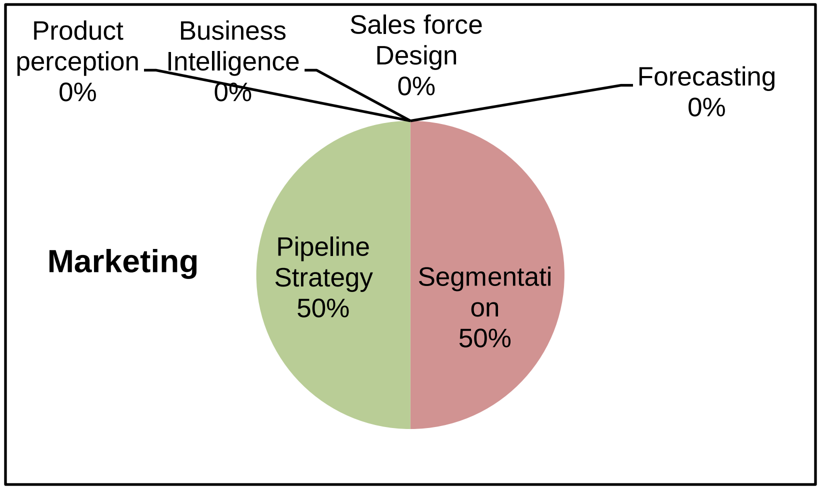
Pfizer with 5 Orphan drugs in pipeline and 8 Marketed Globally was identified as a High priority Company and therefore was placed in the High Pipeline / High Marketed Segment

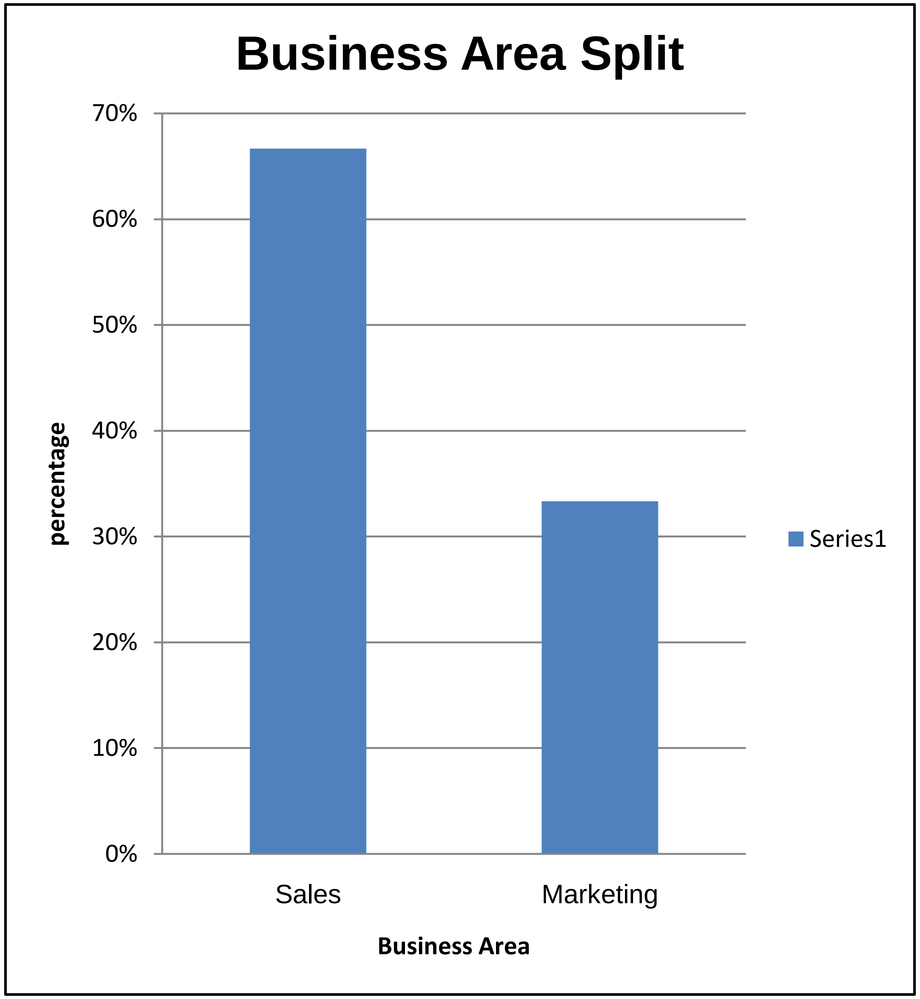




Novartis Pharmaceuticals with 6 Orphan drugs in pipeline and 9 Marketed Globally was identified as a High priority Company and therefore was placed in the High Pipeline / High Marketed Segment







Conclusion

- Shire,Novartis and Pfizer were observed to be investing the maximum for Orphan since they had maximum of number of drug products in development and in market for Orphan indications.
- On creating the unique list of Orphan drugs for different indications, it was identified that the indication Pulmonary Arterial Hypertension had the maximum number of orphan drugs in Market and in pipeline.
- Adalimumab (trade name : Humira) by Abbvie pharmaceuticals for rheumatoid arthiritis was found to be the most successful Orphan drug in Market on taking the overall sales for the particular drug in consideration.
- Afamelanotide (trade name : Scenesse) by Clinuvel Pharmaceuticals for Actinic keratosis was found to be the least succesful Orphan drug in Market on considering the overall sales for that particular product.
- Non- Hodgkin Lypmhoma was observed to have the maximum incidence and prevalance for any Orphan indication