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Executive Summary

BioMarin Pharmaceutical Inc. (BioMarin), is a global biotechnology company established back in 1997 by two research scientists Christopher Starr PhD & Grant W. Denison Jr. from Glyko Biomedical with an investment of a \$1.5 million and went public in 1999 (www.biomarin.com, 2016). The company was further successful in raising \$11.3 million from private investors in the same year. They have offices in the United States of America, South America, Asia & Europe. The primary strategy of the company is developing and commercialising cutting edge treatments for patients who would otherwise go untreated by their afflictions.

BioMarin was the first company to provide therapeutics for mucopolysaccharidoses and phenylketonuria.

Aldurazyme BioMarin's first drug had its first clinical trial in 1997. Aldurazyme is used for the medical care of symptoms of mucopolysaccharidosis I (MPS I) (www.biomarin.com, 1997). In 1999, BioMarin (BMRN) listed on NASDAQ stock exchange (www.biomarin.com, 1999). 2003 granted approval for commercialising Aldurazyme in USA & Europe. This was a monumental breakthrough as Aldurazyme was first “enzyme replacement therapy” drug approved for MPS I (www.biomarin.com, 2003). July 2015 BioMarin listed on NASDAQ 100 (www.biomarin.com, 2015).

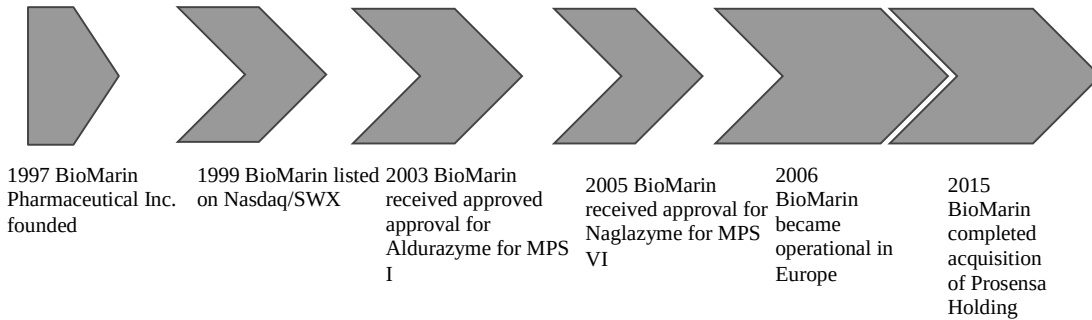
The company's dossier is made up of five commercialised drugs and numerous clinical and pre-clinical drug candidates in R&D. The profit projections of the company can be attributed to their success in the commercialisation of Vimizim™, Naglazyme®, Kuvan®, and Firdapse®;

Aldurazyme® profits are shared equally with Genzyme as it's a trademark of BioMarin/Genzyme LLC. Successful commercialisation of Aldurazyme means they are a pioneer in leading the way for research in enzyme replacement therapies (ERTs).

Products in Clinical Development

Drisapersen, Pegvaliase, Talazoparib, Reveglucosidase alfa, BMN 111, Cerliponase alfa, BMN 044, BMN 045, and BMN 053 (www.biomarin.com, 2016)

BioMarin Timeline



I Current Situation

A. Current Performance

Driver behind BioMarin Valuation?

BioMarin still has not crossed the \$1 billion sales threshold. The company has managed double-digit growth during the past five years. For fiscal 2015, its total revenue stood at \$890 million, up 18.8%. This growth was mainly driven by Vimizim and Kuvan sales.

For fiscal 2016, BioMarin anticipates earnings of ~\$1.05 billion–\$1.1 billion. The advancing pipeline drugs of the company presents huge potential for success to the company. The market capitalisation of the company is \$13.32 billion (www.last10k.com, 2016).

Positive earnings?

Prices in millions

Gross Margin = Gross Profit/Revenue

Gross Profit = Revenue - Cost of sales

(Annual Dec 2015) \$889,895 - \$152,008 = \$737,887

$$\text{Gross Margin} = \$737,887 / \$889,895 = 0.82918 * 100 = 82.92\%$$

1. If the gross margin is > than 40% it shows constant competition dominance
2. If gross margin < 40% revenue diminished by competing companies
3. Gross margin < 20% no dependable competition edge.

$$\text{Net Margin} = \text{Net Income} / \text{Revenue}$$

$$(\text{Annual Dec 2015}) -\$171.799 / \$889.895 = -19.31\%$$

Good indicator of the health, nature & competitiveness of the business but preferred method for measuring for valuation & profitability is net income & earnings per share.

$$\begin{aligned} \text{Operating Income} &= \text{Revenue} - \text{Cost of sales} - \text{Selling, General \& Admin Expense} - \text{R\&D} - \\ &\text{Depreciation, Depletion \& Amortisation} - \text{Others} \\ (\text{Annual Dec 2015}) \quad &\$889,895 - \$152,008 - \$402,271 - \$634,806 - \$47,187 - \$235,675 = - \\ &\$110,702 \end{aligned}$$

$$\text{Operating Margin} = \text{Operating Income} / \text{Revenue}$$

$$(\text{Annual Dec 2015}) \quad -\$110.702 / \$889,895 = -12.44\%$$

↑Operating Margin is more efficient in performance. Has stability during recession or slowdown in industry.

$$\text{Net Income (Annual Dec 2015)}$$

$$= -\$154.724 + -\$17.075 + -\$38.773 + 0 = -\$210.6$$

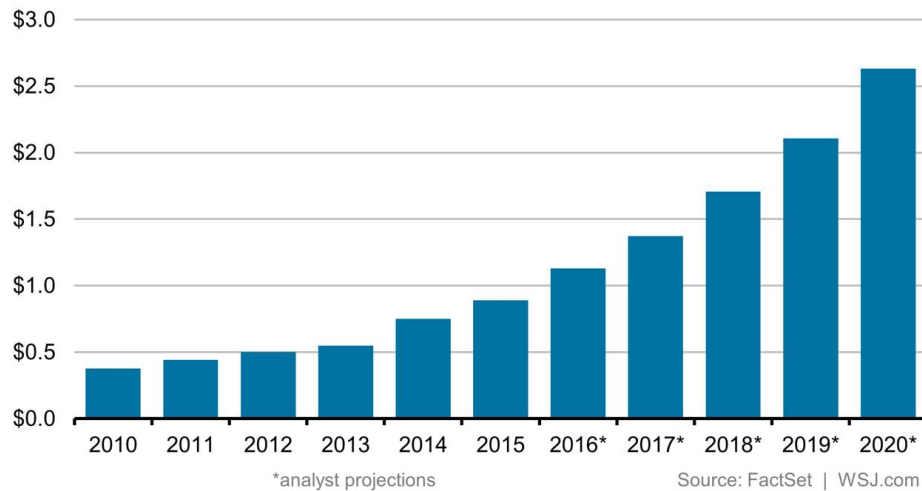
Biomarin's Net Income for the (TTM) ended in Dec. 2015 was -67.501 (Mar. 2015) + -81.989 (Jun. 2015) + -90.926 (Sep. 2015) + 68.617 (Dec. 2015) = \$-171.8 Mil.

$$\text{Earnings per Share} = (\text{Net Income} - \text{Preferred Dividends}) / (\text{Total Shares Outstanding})$$

$$\text{Dec Quarter 2015} \quad \$68.617 - \quad \quad \quad 0 \quad \quad \quad / \$160.025 \quad \quad \quad = 0.43$$

Growth Spurt?

Biomarin annual sales in billions



Graph sourced

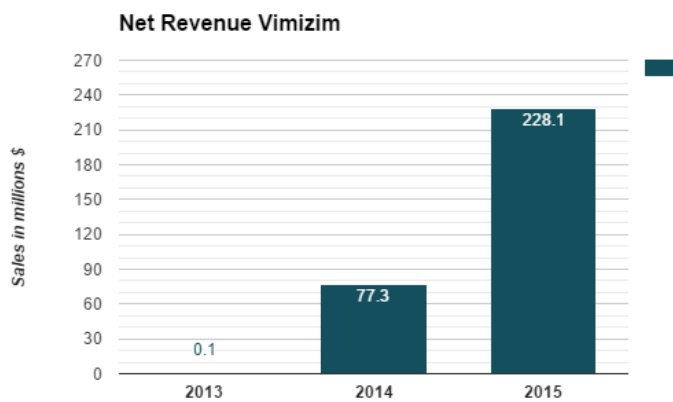
(www.wsj.com, 2016)

BioMarin (BMRN) anticipates a breakeven or profitability in 2017 with rising sales. Pegvaliase and Cerliponase Alfa are two other advanced-stage pipeline drugs that might get permission to be launched in 2017. These along with existing drug portfolio will likely drive BioMarin's revenues across the \$2 billion mark by 2020 (www.last10k.com, 2016).

Sales of Vimizim sore 195% in 2015

Vimizim for mucopolysaccharidosis IV Type A

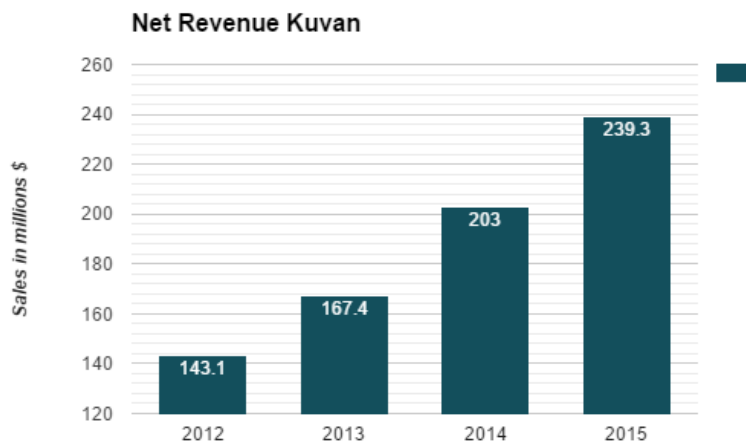
In 2014, Vimizim was awarded market approval by FDA. European Medicines Agency (EMA) followed suit with permission being granted for the medical care of mucopolysaccharidosis IV Type A, or MPS IV A (www.last10K.com, 2016).



Graph created by author using figures from 10K (www.last10K.com, 2016).

Vimizim is a major revenue driver for BioMarin

From the graph, it is clearly evident Vimizim revenue has risen steadily over 2014 sales. It is a major asset to the company generating revenue into the future. Vimizim as a % of total revenues was 25.6% in 2015 compared to 10.32% in 2014. Jump in sales from 2014-2015 up 195% are proof of that success. Vimizim's 2016 revenues expected to earn ~\$300-\$330 million. With regard to the increase in patient demand, BioMarin has been granted approvals for price & reimbursement in Germany, France, Italy, the United Kingdom, Japan & secondary markets. With this procurement of approval, it grants the ability of market penetration for Vimizim (www.last10K.com, 2016).

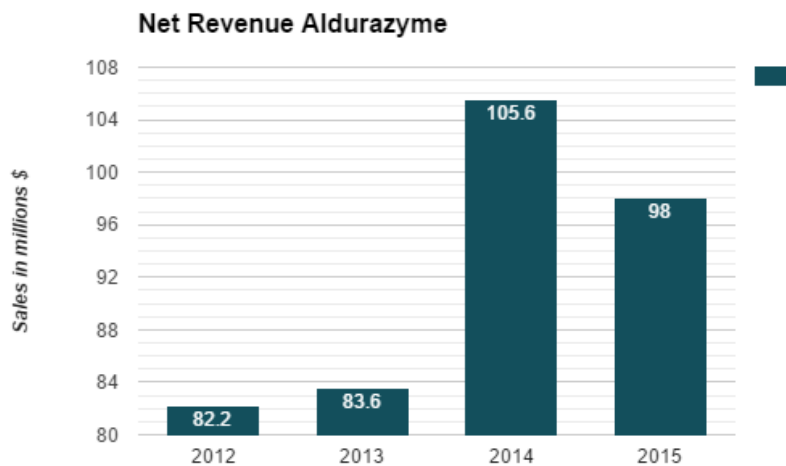


Graph created by author using figures from 10K (www.last10k.com, 2016, 2015, 2014, 2013)

Kuvan and phenylketonuria

Kuvan generated \$239.3 million for BioMarin Pharmaceutical in 2015, which was an increase 17.9% over 2014. Kuvan as a % of total revenue was 26.9%. BioMarin expects Kuvan to fetch \$320 million–\$350 million in 2016. Merck Serono have granted the authorisation of worldwide distribution to BioMarin excluding Japan. The EU has offered exclusivity rights until 2024 & this strategic movement has huge benefit in supporting the growth of the drug. In 2007, the FDA approved marketing in the USA. Following this result EU granted access for sale in Europe. BioMarin markets the drug in the United States & Canada (www.last10K.com, 2016)

Aldurazyme: Biomarin's drug for mucopolysaccharidosis I



Graph created by author

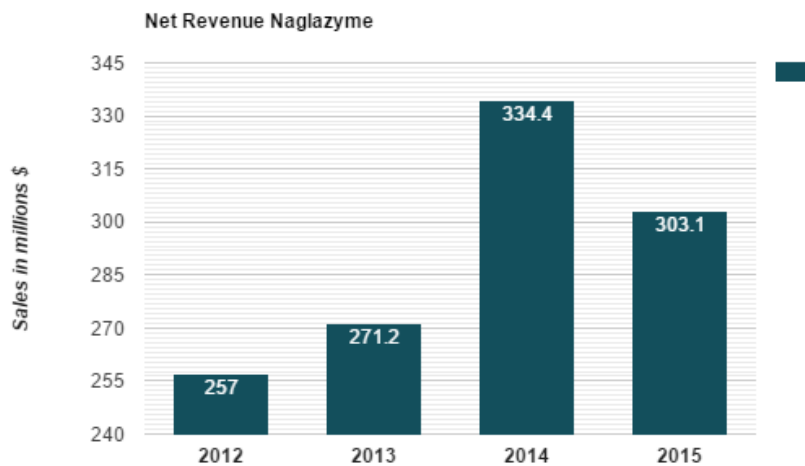
using figures from 10K (www.last10k.com, 2016, 2015, 2014, 2013)

Source Company filings 10K Annual Reports BioMarin 12,'13,'14,'15.

Aldurazyme's revenue increased 1.7% from 2012 to 2013. It moved a further 26% from 2013 to 2014. Finally an obvious decline in 2015 with revenue dropping to \$98 million a decrease of 7.2%. Aldurazyme as a % of total revenue contributed 11%.

Naglazyme: BioMarin's drug for mucopolysaccharidosis VI

Naglazyme (or galsulfase) is an orphan—rare—drug that acquired approval for marketing from the FDA in 2005 for medical assistance of mucopolysaccharidosis VI or MPS VI. In 2006 received approval for sale in Europe, followed by subsequent approval in other countries (www.last10k.com, 2016)

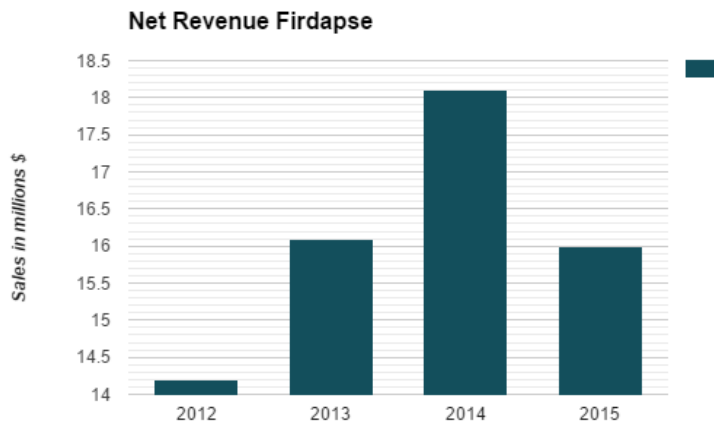


Graph created by author using figures from 10K (www.last10K, 2016, 2015, 2014, 2013)

Source BioMarin 10K Annual Reports Company Filings 12,'13,'14','15

Naglazyme net sales revenue represented in the above graph. For 2015, BioMarin's Naglazyme total revenue was ~\$303 million. Sales were (-31.3). Down 9.4% of ~\$334 million to \$303. Naglazyme % of total revenue is 34%. It has the highest percentile of the total revenue earned for the company. It would be considered driving force behind the revenues earned for the company even though sales dipped in 2015.

Foreign exchange and net hedging has direct impact in lowering the revenue by ~\$30 million. Latin America is the main purchaser of large uneven bulk orders. However, new patients are driving the demand for the drug. Patients on Naglazyme therapy grew by 8.7% in 2015 (www.last10k.com, 2016).

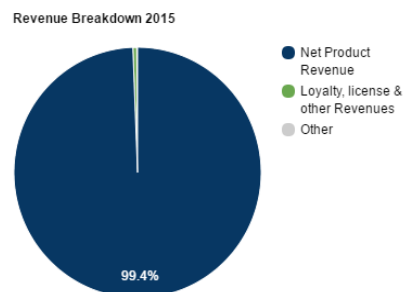


Graph created by author using figures from 10K (www.last10k.com, 2016, 2054, 2014, 2013)

Firdapse has a 13.13% decrease from 2014-2015 from examining the net revenue of Firdapse. Revenue dropped from \$18.1 million in 2014 to \$16 million in 2015. Firdapse has an overall contribution of 1.8% of total revenue.

Graph representation of 3 sources of revenue

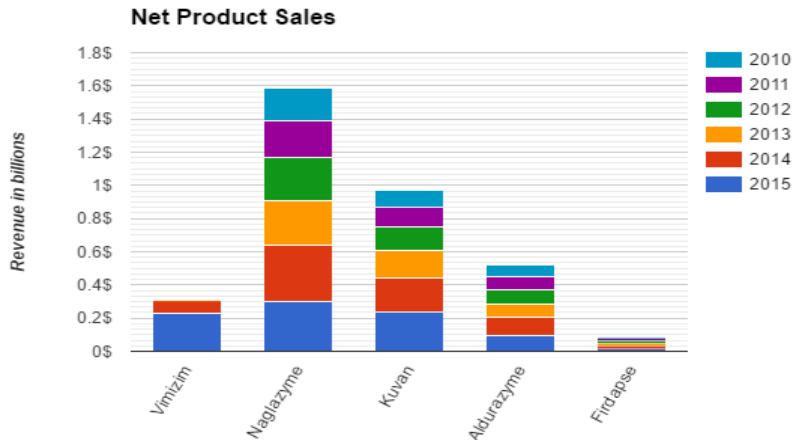
BioMarin (BMRN) acquires their income from 3 sources, namely product revenue, collaborative agreements, as well as royalty, license, and other revenue. The graph below shows the major contribution of net product revenue at ~99% of the company's total revenue. Net Product Revenue \$884.5 million, Collaborative Agreement Revenues \$1.02 million & Loyalty, license & other Revenue \$4.36 million (www.last10K.com, 2016)



Source BioMarin 10K Annual Report for fiscal year ending 31/12/'15 (www.last10k.com, 2016).

Net product revenue

Five commercialised products that contribute to product sales are Vimizim, Naglazyme, Kuvan, Aldurazyme, and Firdapse. The graph below details the revenue earned by the existing product portfolio of the company.



Graph created by author using

figures from 10K (www.last10K.com, 2016, 2015, 2014, 2013, 2012, 2011)

Source BioMarin 10K Annual Reports for fiscal years ending 10'11'12'13'14'15.

B. Strategic Posture

Mission Statement: to use knowledge & research gathered & cultivated to assist patients suffering from genetic disease, severe burn & wounds (www.biomin.com, 2016).

Objectives: BioMarin Pharmaceuticals Inc. are pioneers in sourcing, developing & commercialising therapies that plagued minorities domestically & internationally. “We aim to bring new treatments to market that will make a big impact on small patient populations” (www.biomin.com, 2016)

Strategies: BioMarin's strategy is to organically develop new products; with diverse product line in R&D; target potential merger/acquisitions & successfully be first to market (www.biomin.com, 2016).

First, they need to compete collectively by being seen at all times as *one company, one brand*.

BioMarin has exercised dominance in the biotechnology industry through acquisitions, alliances & collaborations. They have used the strategy of organic growth along with acquisitions to extend its geographical grasp & have the competitive edge with enhanced product portfolio.

1998 BioMarin & Genzyme LLC form 50/50 alliance to develop & commercialise Aldurazyme (www.biomin.com, 2016)

In 2002, BioMarin acquired Glyko Biomedical the company that originally invested \$1.5 million to set it up (www.investors.bmrn.com, 2002).

2005 BioMarin announce strategic coalition with Merck Serono for Phenoptin & Phenylase development & commercialisation (www.biomin.com, 2016)

2007 Collaboration with IGAN for the enzyme therapy advancement to assist treatment of IgA Nephropathy (www.biomin.com, 2016)

2008 BioMarin join forces with Summit Plc. for global licensing agreement of DMD program (www.biomin.com, 2016).

2009 BioMarin partners up in joint venture with La Jolla Pharmaceutical licensing agreement to develop & commercialise Riquent. ® (www.biomin.com, 2016)

2009 BioMarin acquired Huxley Pharmaceuticals Inc. Huxley Pharmaceuticals was a privately held life sciences corporation. Huxley had the rights for the development of Firdapse. This strengthened BioMarin's prominence as it allowed for product expansion (www.biomin.com, 2016).

Firdapse is used predominantly in a number of rare muscle diseases. Firdapse for indication of Lambert Eaton Myasthenic Syndrome (LEMS)

2010 LEAD Therapeutics (www.biomin.com, 2016) is acquired by BioMarin. LEAD carry out specialised research on drug discovery and early stage development with a polyADP-ribose polymerase (PARP) inhibitor. It can be administered orally for treatment of rare, genetically defined cancers.

2010 Marketing approval granted to BioMarin for Firdapse in EU.

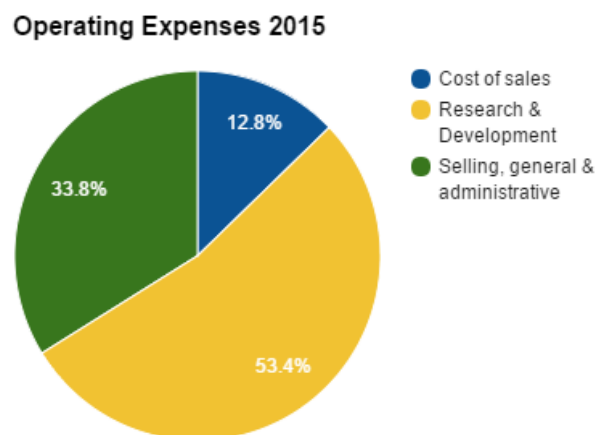
2013 Acquire Zacharon Pharmaceuticals (www.biomin.com, 2016).

2015 BioMarin was successful in acquisition of Prosensa Holding NV (RNA). Securing rights to Prosensa was the driving force behind procurement. BioMarin's main objective was also getting

hands upon experimental drug Drisapersen for alleviating symptoms of Duchenne muscular dystrophy, (DMD), (www.biomarin.com, 2016).

GlaxoSmithKline (GSK) refused to go into partnership with Prosensa after Drisapersen failed phase III clinical trial & handed rights back to Prosensa. GSKs loss was BioMarin's gain as Prosensa discovered the drug was productive in further trials of subjects who were younger & administered doses over longer periods were more responsive. The major advantage to BioMarin is that Prosensa has a lot of data & trials carried out already.

Sarepta Therapeutics (SRPT) are doing their own investigations & work on developing eteplirsen. Both Drisapersen & eteplirsen would be in direct competition for approval from FDA. The 2 drugs are exon-skipping therapies & presently nothing available on market for life threatening DMD. FDA coming under increasing pressure from patients suffering from DMD to grant approval to either drug. It is potentially alluring, beneficial & financially rewarding for major pharmaceutical & biotechnology companies to invest in final stage developmental products (www.last10k.com, 2016).



Graph created by author

from figures obtained from 10k (www.last10K.com, 2016).

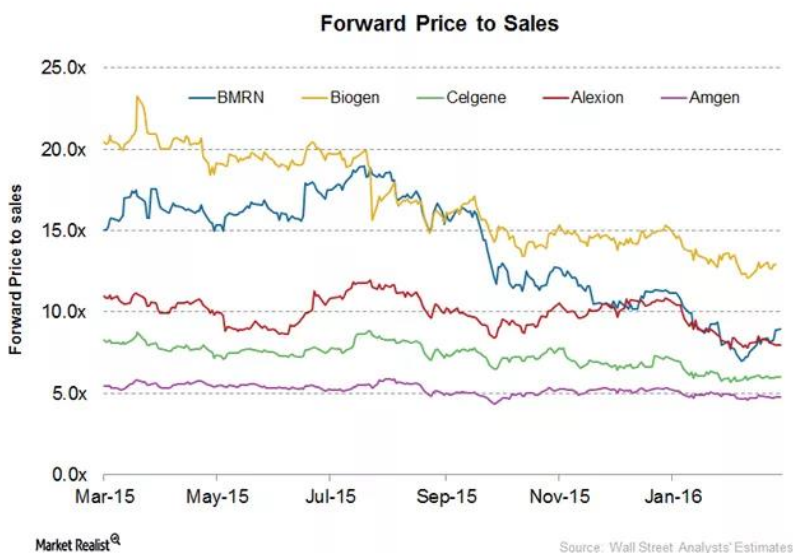
R&D has the largest proportion of the expense for the company. Total operating expenses amounts to \$1.190 billion. Operating expense are outweighing the revenue.

R&D expense

R&D expense was valued at \$634.8 million compared to \$461.5 in 2014. 2015 vs 2014 difference of \$173.3 million. 2013 R&D expense was \$354.8 million. 2014 vs 2013 \$106.7 million. The company reported that higher expense amassed due to continuous clinical activities and a progress of developmental pipeline drugs toward completion.

Projection for R&D expense

BioMarin predictions anticipate the expense to rise by further 10-15% by fiscal year ending in 2016. For 2014 to 2015, the R&D costs of BioMarin was ~71.3% with respect to revenue. It jumped exponentially by ~37% from 2015-2014. Progression in development & spending in initial stage pre-clinical/clinical activity programs are the explanation. Comparing & contrasting BioMarin against its adversaries such as Biogen (BIIB) Amgen (AMGN) and Celgene (CELG), we can identify BioMarin's expense as too high. Closest competitors' sales lie in the value range of 19-31% (www.last10K.com, 2016.) This could be equated to why BioMarin's revenue base is lower & is counterbalanced by expense of more pipeline drugs for advancement. With increase in demand for drugs, revenue base will rise & expense as a percentage of sales will start to fall or hopefully level out in coming years.



Source Market Realist

(www.marketrealist.com, 2016).

Valuation multiples for BioMarin

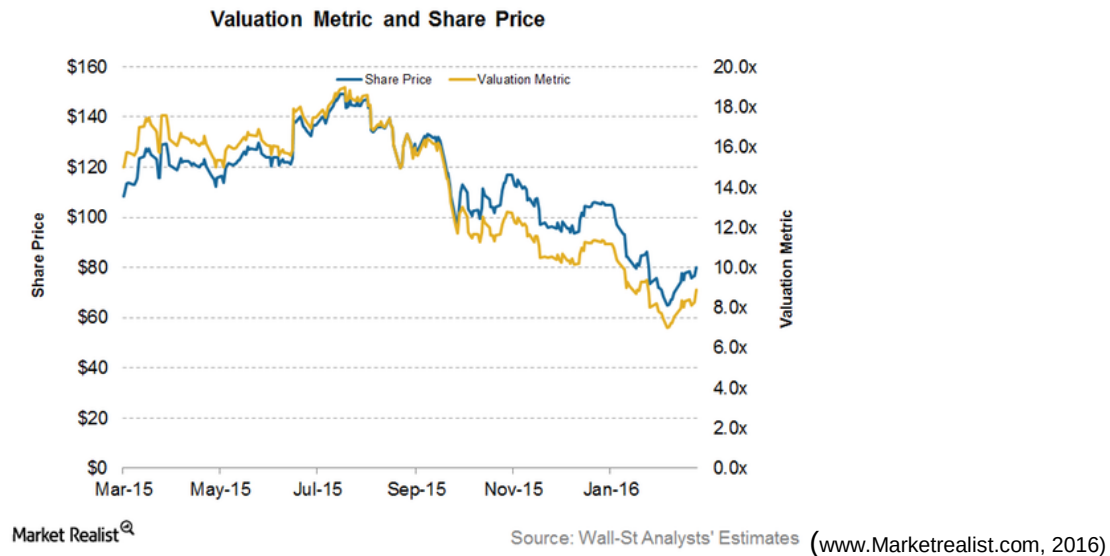
Price to Sales Valuation

Selling, general, and administrative expense

Selling, general, and administrative expense forms ~45.2% of BioMarin's total functioning costs/expense. With the likelihood of more market penetration activities for Kuvan and Vimizim in the future, the company is experiencing a rise in this expense. From the balance sheet for fiscal year ended 31st of December 2015, SG&A expense was \$402 million. It has risen steadily by ~100 million each year. So expect 2016 SG&A to be ~\$505 million mark. In 2016, BioMarin anticipate Kuvan net product revenues to increase over 2015 levels through their joint venture/agreement with Merck Serono to acquire global distribution to Kuvan and Pegvaliase, omitting Japan, officially came into effect January 1, 2016. The gross margin of Kuvan is not

envisaged to change dramatically in years to come. However, we expect to see generic competition for Kuvan in the future. Also, the company predicts a higher expense given the potential launch of Kyndrisa (www.last10K.com, 2016).

Recent fall in the valuation metric



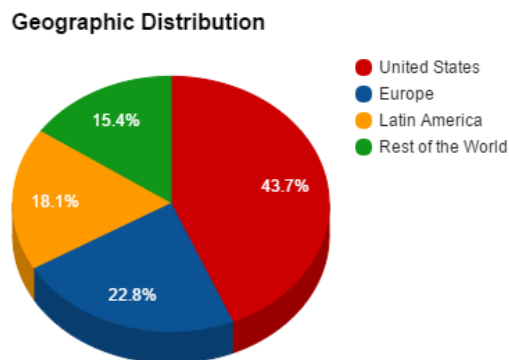
Cost of Sales

Total cost of sales for the years ended December 31, 2015 was \$152.0 million and \$122.3 million in 2014. The increase in cost of sales was primarily attributable to the increase in product sales (www.last10k.com, 2016).

R&D Failure Is the Principal Risk Faced by BioMarin

Risks

The biotechnology industry is a highly competitive & volatile environment. It operates on a highly dynamic level continuously trying to develop new products. The principal risk faced by the industry is R&D (research and development) failure risk. BioMarin is contending with a variety of factors such as fluctuating currencies, foreign currencies & credit risks.



Graph created by author from figures

10k (www.last10k.com, 2016)

Currency fluctuation risk

The graph above presents the geographic revenue distribution for BioMarin. The company derives ~44% of its net product revenue from the United States. The remaining 56% is earned from international markets, and the company faces currency fluctuation risk. A strong dollar against a foreign currency would result in falling revenue for the company (www.last10k.com, 2016).

Credit risk

For fiscal year ending December 2015, two of the largest customers of the company held 31% & 17% of the total receivable balance from net Vimizim, Naglazyme, Firdapse and Kuvan product revenues. This exposes the company to the risk arising from default (www.last10k.com).

R&D failure risk

Pharmaceutical or biotechnology companies are always trying to attain rights to pipeline drugs, as they could be potentially very lucrative in the long run. BioMarin's revenue for 2015 was ~\$889 million, the company's market capitalisation as of March 20, 2016, is ~\$13.32 billion. This correlates to promising expectations enroute concerning pipeline products coming into development & existence to the market if approved by the licensing authorities. BioMarin has plans & intentions to launch 3 drugs in 2016. Kyndrisa for Duchenne muscular dystrophy (DMD). Pegvaliase for phenylketonuria (PKU) & Cerliponase alfa for CLN₂ disorder a late infantile form of Batten disease (www.last10k.com, 2016).

BioMarin Policies

BioMarin's global Corporate Compliance & Ethics Program (CCEP) is an enterprise wide global initiative that addresses the seven elements that are discussed in the Office of the Inspector General (OIG) Compliance Program Guidance for Pharmaceutical Manufacturers, as well as the tenets of the U.S. Federal Sentencing Guidelines, and other applicable laws and regulations.

- 1) Implementing written policies & procedures.
- 2) Designating a compliance officer & compliance committee.
- 3) Conducting effective training and education.
- 4) Developing effective lines of communication
- 5) Conducting internal monitoring and auditing.
- 6) Enforcing standards through well-publicised disciplinary guidelines.
- 7) Responding promptly to detected problems & undertaking corrective action.

The purpose of the CCEP is to prevent, detect and correct fraud, misconduct, and violations of company policies, procedures, and/or applicable laws and regulations. The CCEP applies to BioMarin Directors, officers, employees and, in certain situations, agents, consultants, and independent contractors hired by the company.

Oversight:

The Corporate Governance & Nominating Committee and Audit Committee of the BioMarin Board of Directors has ultimate authority to oversee the CCEP, Corporate Compliance & Ethics Officer (the "Compliance Officer") and Corporate Compliance Committee (CCC). The CCC is comprised of executive members from functional units across the company. The intent of the Board and company officers is to set the tone for the ethical behaviour expected of all Directors, officers, employees, and business partners (www.files.shareholder.com, 2016), (www.oig.hhs.gov, 2003).

Corporate Governance

Board of Directors - Overview



Elaine J. Heron, Ph.D. Age 67
Board member since July 2002. 13 years in service.
B.S.C in Chemistry, PhD in Analytical Biochemistry.



Pierre Lapalme. Age 74
Board member since January 2004. 11 years in service.
Attended INSEAD France and the University of Western Ontario.
Shares 31,250



Michael Grey Age 62
Board member since December 2005. 10 Years in service.
B.S.C in Chemistry, University of Nottingham, United Kingdom.



Jean-Jacques Bienaimé, Chairman and Chief Executive Officer. Age 62
Board member since 2005. 10 years in service.
M.B.A. Wharton School, Pennsylvania. Economics Degree École Supérieure, Paris.
Shares: 417,979



Alan J. Lewis, Ph.D. Age 70
Board member since June 2005. 10 Years in service.
B.Sc. in physiology & biochemistry. Ph.D. in pharmacology.
Shares: 22,500



Randy Meier - Lead Independent Director Age 56
Board member since December 2006. 9 Years in service
B.E Economics
Shares 25,250



V. Bryan Lawlis, Ph.D. Age 63
Board member since June 2007. 8 Years in service.
B.A. in microbiology and a Ph.D. in Biochemistry.
Shares: 11,850



Dennis J. Slamon, M.D., Ph.D. Age 66
Board member since March 2014. 2 years in service.
B.A. Biology. Ph.D. in cell biology
Shares: 6,325



David Pyott Age 62
Board member since January 2016. 0 years in service.
Diploma International & European Law, Master of Arts Degree & M.B.A
Shares 2,100

The Board of Directors

The Biomarin board of directors comprises eight independent directors and one chairman director. The average age is 65, the youngest 56, the oldest 74.

The board oversees the business and is actively involved in the oversight of risk to the company. It carries out this function through four committees, each reporting to the board on a regular basis and has direct access to management, especially to those officers responsible for particular risks. It establishes policies and evaluates the accomplishments of senior managers.

In the past twelve months, two directors have resigned from the board.

Mr William D. Young resigned in November, not citing a reason and Mr Kenneth M. Bates resigned in December due to a conflict of interest in his other activities. Both parties confirmed they had no dispute with the company.

In January, David Pyott was elected to the board.

The current board spans a great wealth of knowledge across the pharmaceutical and biotechnology industry. It could be described as diverse and technical in regards competences held.

Five of the members have been involved in the sale or acquisition of public and private companies, having either founded or presided over them until sale or acquisition.

Seven members have held presidency and CEO positions and two have held Director and CEO positions.

Two members have significant research experience, one having dedicated 20 years to cancer research and the other published 120 manuscripts and written and edited seven books.

Stated in the 2015 Proxy report, *Investors.bmrn.com. (2016)*, the board made a decision to recombine the Chair of the Board of Directors and Chief Executive Officer, whereby Pierre Lapalme steps down as current chair and Jacques Bienaimé takes over both positions. Being the only employee director, Jacques Bienaimé does not receive payment for his services to the board. In some countries for example, Germany, the Netherlands and Finland, it is viewed that in combining the position, the CEO cannot effectively preside over top management if he is part of top management.

To offset the imbalance in power, a lead director is often nominated to coordinate the CEO evaluation among other duties.

Biomarin have done exactly this. In conjunction with Jacques Bienaimé taking over both positions as CEO and Chair, Richard Meier was elected to the position of Lead Director.

He has a number of responsibilities ranging from shareholder engagement, organising board meetings, presiding in the absence of the chairman and also to act as liaison between the CEO and the independent directors.

The CEO is annually reviewed by the board of directors through a structured nominating committee, which prevents the CEO from electing directors more passive to his proposals.

The board's expertise is administered through four committees. These are the Audit, Corporate Governance & Nominating, Science & Technology and Compensation committees.

To avoid a conflict of interest the chairman of the board, who must also hold the position of CEO, does not participate on any of these committees.

The committees oversee the overall functioning, strategy and direction of the business.

Each committee nominates a chair, all members must be independent as that defined by NASDAQ and, if applicable, the Sarbanes-Oxley Act of 2002. The Audit committee must meet four times per year, whilst the others meet at least two times per year. The science & Technology committee must have at least two directors, whilst the other committees must have at least three. The Science & Technology committee has the highest membership at five members, strategically placing the members with the least scientific and technology background on the other committees.

The Audit committee is chaired by Randy Meier most likely for his specialist skills in finance. This committee nominates the external independent financial auditors (in this case KPMG) and are responsible for their compensation and retention. The independent auditors report directly to the committee, whilst the committee engage both management and the auditors as to the completeness and accuracy of the accounts. Management and auditors are ultimately responsible for their own financial records and declarations.

Before the release of the “*Management’s Discussion and Analysis of Financial Condition and Results of Operations*” on the annual K-10 SEC report, the committee will engage both management and auditors before release of such information.

Chaired by Elaine Heron. PhD, the Corporate Governance & Nominating Committee are responsible for overseeing that suitably qualified people are elected to the board and are in compliance with The NASDAQ Stock market LLC and the Securities and Exchange Commission.

In relation to the operation of the board, its committees and management, it oversees the development and implementation of corporate principles and policies relating to codes of conduct and ethics. It evaluates compliance and recommends training and induction for new directors where necessary. As mentioned earlier this committee oversees the election and succession planning of the company CEO, thus avoiding the likely hood of the board being unduly influenced by the CEO.

The Compensation committee oversees the development and implementation of compensation programs. It consults with senior management as to a general compensation philosophy and makes recommendations to the board.

It evaluates the performance of the CEO and recommended compensation adjustments accordingly.

It reviews the general incentive compensation plans, equity based plans and share incentive plans.

It also recommends to the board corporate goals and objectives relevant to the compensation of the CEO’s direct reports and other senior management as appropriate.

The Science & Technology committee assist the board when considering senior management decisions regarding company operations. This covers the deployment, allocation, utilization and investment of scientific assets.

It provides the board with an oversight into the companies IP, Technology and Strategy to help the decision making progress in regards scientific programs.

This appears to be very much a hands on approach as the board “*review and considers*” management decisions regards technology acquisition/divesting, or investment in research programs. *Investors.bmrn.com. (2016).*

The committee is chaired by Dr. Alan Lewis and is composed of the five most scientifically qualified and experienced directors of the board. The remaining three are characterised by their business acumen over their technical prowess.

Board shares and incentives – Agency Theory

Investors.bmrn.com. (2016). Insider Ownership

Investors.bmrn.com. (2016). BioMarin Pharmaceutical Inc.

Director	No. of shares	Value @81.9 \$USD
Jean-Jacques Bienaimé	417,979	\$34,232,480.00
Elaine J. Heron	35,625	\$2,917,687.00
Pierre Lapalme	31,250	\$2,559,375.00
Michael Grey	26,250	\$2,149,875.00
Randy Meier	25,250	\$2,067,975.00
Alan J. Lewis	22,500	\$1,842,750.00
V. Bryan Lawlis	11850	\$970,515.00
Dennis J. Slamon, M.D., Ph.D.	6325	\$518,017.00
David Pyott	2100	\$171,990.00

Directors, executives and senior management are required to hold certain amounts of stock directly in Biomarin, to help align their personal interests with those of the shareholders. The amounts for each band are as follows

Non-Executive Directors – Stock value equal to or less than 10,000 shares. Or three times their cash retainer value.

CEO - Three times base salary.

Executive Vice president and Senior Vice presidents – Two times base salary.

Agency theory as defined by *Wheelen, T. and Hunger, J. (2000)*, states that directors and top management should own a significant amount of stock so as to align their personal interests with those of the shareholders. In other words, management will make decisions for the company, which will leverage growth in its share price, thereby increasing their own personal wealth as well as that of the shareholders. It needs to be adequately significant to ensure the desirable effect.

Biomarin very much adheres to this theory. Their regulations specify how much stock is required to be owned by top management and directors.

The market capitalization of Biomarin has grown from \$450million in 2005 to \$15billion in 2015, indicating a positive correlation to agency theory.

Codetermination, the practice of having employees serving on boards, is not practised at Biomarin. The only employee director on the board is the CEO and he is not compensated for his role on the board.

The K-10's, reported to the Security and Exchange Commission, state that none of the existing directors have an affiliation with senior management or employees to avoid a conflict of interest in regards compensation, policies, ethics etc.

Board of Directors – Summary

This board is populated by some of the most highly qualified, experienced and extremely astute business people in the pharmaceutical and biotechnology industries. In just under 10 years, Jean Jacques Bienaimé has brought this company from a \$450million market capitalization company to a \$15billion market capitalization company and received a number of awards. Dr. Alan Lewis, Michael Grey, V. Bryan Lawlis PhD not only have very significant qualifications and expertise, but they also have a wealth of experience starting and developing pharmaceutical/biotechnological companies. In many cases, these companies were either acquired or sold, demonstrating their ability to build successful businesses. David Pyott was listed fourth in Harvard Business Reviews top 100 CEO's *Harvard Business Review*. (2014). David took Allergan from \$1billion in sales to \$7billion in sales between the years 1998 and 2014. "*He refocused Allergan's strategy on specialty pharmaceuticals as well as restructuring its worldwide operations.*" *Allergan, I. (2016)*, demonstrating the breadth of his international experience and capability. Pierre Lapalme has strong international experience rolling out ethics programs in North and South America and was a member of the Pharmaceutical Manufacturers Association of Canada. He also played a leading role in reinstituting patent protection for pharmaceuticals, experience highly valued at Biomarin due to their niche market strategy. Given his specialty in cancer research, the appointment of DR. Slamon would have been of significance for the development and sale of Talazoparib to Medivation, a drug for the treatment of metastatic breast cancer. He has spent a significant amount of his career researching treatments for cancer, specifically breast cancer, where he played a major role in the development of Herceptin, a drug that dramatically improves the lives of many women suffering from breast cancer. His work has been recognised with many awards for his contribution to the field of cancer research.

(See Appendices 1 – 4 for Board of Directors background synopsis.)

Top Management - Overview



Robert A Baffi, Executive Vice President Technical Operations
Age 60. Joined 2000. 15 years served.



Joshua A. Grass, Senior Vice President, Business & Corporate Development
Joined 2002. 13 years served.



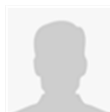
G. Eric Davis, Executive Vice President and General Counsel
Age 44. Joined 2004. 11 years served.



Jeff Ajer, Executive Vice President and Chief Commercial Officer
Age 52. Joined 2005. 10 years served.



Jean-Jacques Bienaime, Chairman and Chief Executive Officer
Age 61. Joined 2005. 10 years served.



Scott Clarke, Senior Vice President Product Development
Joined 2005. 10 years served.



Philip Lo Scalzo, Senior Vice President, Chief Compliance Officer
Joined 2007. 8 years served.



Eduardo E. Von Pervieux, Vice President Information Technology, Chief Information Officer
Joined 2008. 7 years served.



Dr. Henry J. Fuchs, Executive Vice President and Chief Medical Officer
Age 57. Joined 2009. 6 years served.



Dan Spiegelman, Executive Vice President and Chief Financial Officer
Age 56. Joined 2012. 3 years served.



Richard Ranieri, Executive Vice President, Human Resources and Corporate Affairs
Age 60. Joined 2013. 2 years served.

Top Management

Almost fifty percent of top management have worked at the same two companies Genentech and Genencor. This includes the company CEO, Jacques Jean Bienaimé.

According to the March 20th 2015, Proxy statement, *Investors.bmrn.com. (2016). BioMarin Pharmaceutical Inc.*, there are seven executive officers included Mr. Bienaimé and Mr. Mueller, who is not listed on the company website as an executive director.

There are twelve members of top management, seven listed as executives, three senior vice presidents and one vice president.

The average age of the executives is 54, the youngest 41 and the oldest 61.

Two managers have joined in the past three years, while fifty percent have worked for more than ten years at Biomarin and none were employed by companies acquired by Biomarin.

Robert A Baffi, Executive Vice President Technical Operations, aged 60, joined Biomarin in 2000, with 15 years served.

He is also on the board of Directors at Kalobios with BioMarin's Non-Executive Director, Dr. Alan J Lewis. He has considerable expertise in Quality and oversees manufacturing, process development, quality & analytical chemistry at Biomarin. He previously worked at Genentech and is qualified to a Ph.D. MPhil, & B.S. Biochemistry level.

Joshua A. Grass, Senior Vice President, Business & Corporate Development.

He joined in 2002 and has currently served 13 years. His expertise is prominently dealing with financial relations and has raised \$1billion in capital for Biomarin. Considering his current role, he interestingly holds a B.S. in Biology.

G. Eric Davis, Executive Vice President and General Counsel. Age 44 with eleven years served.

Jeff Ajer, Executive Vice President and Chief Commercial Officer. One of the first sales & marketing employees at Biomarin and has since been instrumental in developing their commercial operations. Genzyme was a previous employer.

Scott Clarke, Senior Vice President Product Development has been working with Biomarin for 10 years. He oversees product pipeline, alliance formations and acquisitions. He led the development of a drug device combination with PhotoBioChem BV, holds an M.B.A, a B.S. Chemical Engineering and a B.A. Biotechnology.

Philip Lo Scalzo, Senior Vice President, Chief Compliance Officer has worked with Biomarin for eight years and holds responsibility for compliance including audits, ethics and corrective actions.

Eduardo E. Von Pervieux, Vice President Information Technology and Chief Information Officer. Strong experience implementing global IT systems for supply chain technologies.

Dan Spiegelman, Executive Vice President & Chief Financial Officer. Age 56 and has currently served three years. Previously employed at Genentech.

Richard Ranieri, Executive Vice President, Human Resources and Corporate Affairs. Currently at Biomarin two years and previously employed by Genencor.

Dr. Henry J. Fuchs, Executive Vice President and Chief Medical Officer. Age 57. Currently with Biomarin six years. He led the program that approved Pulmozyme, for the treatment of Cystic Fibrosis, when working at Genentech.

Mr. Brian R. Mueller, Group Vice President, Corporate Controller and Chief Accounting Officer. He is the youngest director at 41 years of age and joined Biomarin when he was 28 years old. He holds a B.S. in Accountancy, awarded by the Northern Illinois University.

Top management share ownership

Investors.bmrn.com. (2016). Insider Ownership

Investors.bmrn.com. (2016). BioMarin Pharmaceutical Inc.

Manager	Title	No. of shares	Value @ 81.9 \$USD
Jean-Jacques Bienaimé	CEO	417,979	\$34,232,480
Robert A Baffi	Executive	220,895	\$18,091,300
Joshua A. Grass	Senior VP	Unknown	
G. Eric Davis	Executive	91,480	\$7,149,212
Jeff Ajer	Executive	59,030	\$4,834,557
Scott Clarke	Senior VP	Unknown	
Philip Lo Scalzo	Senior VP	Unknown	
Eduardo E. Von Pervieux	VP	Unknown	
Dan Spiegelman	Executive	63,933	\$5,236,112
Richard Ranieri	Executive	Unknown	
Dr. Henry J. Fuchs	Executive	120,666	\$9,882,545
Mr. Brian R. Mueller	Executive	15,247	\$1,248,729

As described earlier, Executive Vice presidents and Senior Vice presidents must hold twice their base salary in stock ownership, so as to align interests of their personal wealth with that of shareholders.

Top management - Summary

With half of top management having been with the company for over ten years, they are extremely familiar with their roles and the environment they operate within. The 2015 proxy details three of their highest earners being compensated in the range of \$2.6 to \$3.2 million USD annually. A very significant motivating factor.

Their experience and qualifications address the needs of the company, over its various functions very well. The team holds a very good balance between youth and experience, thereby providing a stable future for the company. In combination with an older and wiser board of directors, the younger top management will benefit greatly from their experience, serving the company further by laying strong foundations for its future.

(See Appendices 5 – 7 for Top Management background synopsis.)

Social Responsibility at Biomarin

Biomarin have implemented a Global Code of Conduct and Business Ethics program. Each new employee is inducted through this program. The policy describes principles and standards to be adhered to when conducting business on behalf of the company and specifically outlines each affected area of the company, from the employee to the external environment the company operates within. It describes an ‘Open Door’ policy whereby employees may ask a question, present an idea or raise a concern, especially those regarding a legal or ethical matter. The code covers many other topics such as bribery and corruption, how to deal with suppliers, healthcare professionals, human and animal welfare.

The board has a very practical approach to caring for the environment and sustainability. At the 2015 shareholder meeting, recorded in the 2015 proxy *Investors.bmrn.com. (2016). BioMarin Pharmaceutical Inc. - Definitive Proxy Statement*, shareholders proposed the company start tracking and reporting environment, social and governance (ESG) metrics in accordance with the Global Reporting Initiative (GRI). The shareholders made a case that other companies in the industry are participating and so should Biomarin.

The board rejected the proposal and encouraged shareholders to vote ‘No’ on the basis the benefits do not outweigh the cost.

They also say the companies compared against them were many times bigger and could afford such reporting programs.

They emphasise they are not opposed to improving ESG matters and actively, improve ESG concerns in a practical way by making their buildings LEED certified, improving production processes by reducing water wastage and reusing energy through the purchase of generators designed to co-generate energy and use the exhaust heat to create steam and hot water. Biomarin are a relatively new company and therefore are in a strong position to purchase building & equipment that can deliver energy efficiency and sustainability.

III External Environment: Opportunities & Threats

A. Natural Physical Environment: Sustainability Issues

BioMarin manufactures in-house and employs the use of outsourced contractors. The company has two directly owned manufacturing facilities, in Novato, Southern California and Shanbally, Cork, Ireland. The Novato facility is the only facility licensed to manufacture Vimizim®, Naglazyme® and Aldyrazyme® and lies in the San Francisco bay area, a known earthquake zone. The company is at risk of not meeting commercial and patient needs of these products if the Novato facility is damaged or production is interrupted. There is a need to gain FDA/EMA manufacturing licenses for these products either in Ireland at the Shanbally facility or with outsourced manufacturing contractors to ensure product availability in the event of a major disruption at the Novato facility.

The threat of other natural disasters (flooding, lightning, fire) or acts of terrorism/criminality are of no higher risk significance for any of BioMarin's facilities/properties (see Table 1 STEEP analysis, Ecological factors).

BioMarin actively participates in projects to improve the sustainability of its surrounding environment and communities by adopting initiatives to contribute to and protect the environment. They have installed new equipment in the Novato facility to reduce water usage, installed e-car charging stations at all facilities and offices, and installed solar panels on all building and garages with unrequired generated power sold to state power grid (www.BioMarin.com, 2016). The company intends to roll out similar projects and initiatives in its Shanbally facility.

B. Societal Environment

Table 1. STEEP (Sociocultural, Technological, Economic, Ecological & Political/Legal) ANALYSIS OF TRENDS IN THE EXTERNAL ENVIRONMENT OF BioMarin

(Wheelen & Hunger, 2008)

Trend #	Sociocultural	Technological	Economic	Ecological	Political/Legal
1	Education	IP & Patent Protection	R&D tax credits	Recycling & waste disposal	Foreign Trade Regulations
2	Demographic Hubs	Alternative energy sources	EU Gateway for distribution	Carbon footprint	Corporate Tax rate
3	Skilled labour force	Transport infrastructure	Foreign exchange rates	Sustainable energy	Industry Regulations
4	Available labour force	Telecommunication infrastructure	Healthcare Budgets	Global warming Impact	Health & Safety law
5	Health & Safety law	Internet availability	Corporate tax rates	Natural Disaster threats	Political environment
6	Birth Rates	Technology transfer regulations	Tax treaties	Environmental impact	Environmental Legislation
7		R&D Expenditure	Inflation Rates		Employment Law
8			Biosimilars (generics)		Corporate Governance
9			Outsourcing capabilities		Corporate Social Responsibility
10			Niche Markets		
			R&D Expenditure		

Table 1 above details the environmental factors in the biopharmaceutical industry and the world at large that are considered to be influencing how BioMarin is operating and which may affect future plans for the organisation. These environmental factors were then investigated in terms of their opportunity or threat when considered from the viewpoint of the stakeholders or interested parties who exist in BioMarin's external environment.

The environmental factors in terms of opportunities and threats for BioMarin currently and in the future are:

SOCIOCULTURAL – The benefits that the company can acquire from being located in regions where international biopharmaceutical hubs exist such as manufacturing in San Francisco, US and Cork, Ireland, global commercial organisations in Ireland and other international regions will enable innovation and empower companies due to the education structure that is already in place to provide graduates and skilled personnel to work in this highly regulated environment. The benefits to the local stakeholders – local communities, suppliers, etc. are in available employment in an industry that is evolving rapidly and wages that are considered above average, and due to the investment costs locating manufacturing facilities, there is a reasonable future stability to the positions and spendable incomes.

TECHNOLOGICAL – As above the local stakeholders in areas where biopharmaceutical hubs exist gain opportunities due to the technological requirements in terms of infrastructure – transport, telecommunications and internet availability – that the industry requires. For BioMarin it is also an opportunity due to the location of their operations where these infrastructures are already present.

ECONOMIC – BioMarin locating to Ireland with a global commercial centre in Dublin, Ireland and a manufacturing facility in Cork, Ireland has opened multiple opportunities for the company's future financial strategies with the ability to take advantage of a corporate tax rate of 12.5 % and R&D tax credits of 25% (IDA, 2016) and a stable region in which to do business.

There are also threats in the economic environment with the risk of biosimilars (generic) products entering the global biopharmaceutical market in direct opposition to BioMarin. At present, the threat to BioMarin's revenue stream is minimal but this could be a major threat in the future if BioMarin fails to advance their R&D pipeline with commercially viable drugs to replace their current patented products.

Threats also come in the form of healthcare budgets in all regions of the market. BioMarin's products are expensive with Vimizim® treatment costing US\$ 380,000 (Staton, 2014). Patients cannot afford these prices so there is pressure on healthcare agencies in Europe and in the US, government and insurance programs to cover the major cost of the drug treatment.

POLITICAL/LEGAL – The opportunities for BioMarin in its locations of operation exist in the historical legislation present in terms of Health & Safety and Employment Law. The US and Ireland have proven records in their vigilance of the laws and the regulations that companies must adopt. Strong emphasis on Corporate Social Responsibility and actively encouraging companies to participate in their local communities is seen as an opportunity for BioMarin in its Irish operations. The roll out of its current sustainability programs in Ireland, that it adopts in US, will benefit the local environment but also benchmark the BioMarin caring for the local environment – reducing water usage, generating power from solar energy, etc. (www.BioMarin.com, 2016)

The threats that BioMarin are presented with in the political/legal environment are led by pharmaceutical legislation and the regulators who oversee their implementation. The journey from innovation to commercially available drug product is costly and time consuming with costs of US\$2.6 billion over an average 10 years (www.PhRMA.org, 2015) before a return on investment is achieved.

C. Task Environment

(a) Threat of new entrants

The entry barriers for new entrants into the biopharmaceutical market are extremely high. R&D costs in the region of US\$2.6 Billion for each drug and high percentage failure in taking a new drug to market with only 12% of drugs entering clinical trial successfully entering the market are staggering investments with likelihood of return taking up to 10 years (www.PHRMA.org, 2015). Regulatory approved biopharmaceutical drugs require manufacturing facilities costing in the region of US\$ 900 million (Stack, 2014). The costs of exiting the market are as high due to past investments in facilities, equipment and staff unless the company is incorporated in a takeover or becomes a subsidiary of another company; the barriers to exit are immense.

Conclusion: Threat of new entrants to the future of Biomarin are **LOW** due to high entry and exit barriers in the industry.

(b) Bargaining power of buyers

Biomarin produce patented medicines that are expensive and are required by some patients over a lifetime as they control symptoms of disease rather than a disease cure. As such they are prescribed by medical doctors who cannot always use their own discretion in what they advise or prescribe. Influenced by insurance companies (US) or healthcare providers (EU) in what costs a company or state body will accept, doctors cannot always prescribe the best drug treatment in terms of rare diseases due to limitations on what is approved in that country, healthcare region or medical insurance company. In 2011, the East Midlands Specialised Commissioning Group advised that NHS general practitioners should not prescribe Firdapse® due to cost (East Midlands Specialised Commissioning Group, 2011).

Conclusion: Bargaining power of buyers is **HIGH** and has immense influence on the prescribing and availability of Biomarin product

(c) Threat of substitute products or services

The Falsified Medicines Directive issued in 2011 by the European Commission laid down guidelines for MAH (Marketing Authorisation Holders) and WDAH (Wholesale Distribution Authorisations Holders) on the future requirements that had to be met to ensure the integrity of the pharmaceutical supply chain and the safety and efficacy of medicines both in Europe and in distribution channels throughout the rest of the world (European Commission, 2011). Patent protection and Intellectual Property (IP) Rights are stringently upheld in developed countries with heavy fines if patent or IP rights have been infringed. The need to uphold patents and IP rights are

highlighted by the PHRMA and the need for patent and IP protection to allow further innovation by biopharmaceutical companies in the future (Grayson, 2013).

Conclusion: Threat of substitute products is **LOW** for Biomarin as their drugs are under patent and IP protection, but the threat will increase as patent exclusivities end.

(d) Bargaining power of suppliers

Due to the nature of biopharmaceutical manufacturing, the need for multiple raw materials and in large quantities is not required as manufacture is based around the synthesis of live cells to produce the drug.

Biopharmaceutical manufacturers have therefore fewer suppliers who they are dependent upon to maintain supply to manufacturing lines. What is important is the supply of qualified and competent employees. Due to the rapid expansion of biopharmaceutical companies both in the US and Ireland the demand for qualified personnel could exceed the availability of key personnel in the future. In Ireland we have a dedicated training organisation in the National Institute of Biopharmaceutical Research and Training (NIBRT) which is funded by the government and biopharmaceutical companies (Stanton, 2016) to supply qualified and competent personnel to the industry whether they have graduate qualifications in the industry or not.

Conclusion: Bargaining power of suppliers is considered to be **MEDIUM** as Universities/Colleges etc. are providing biopharmaceutical courses and NIBRT and other institutes are providing training.

(e) Rivalry among competing firms

BioMarin received bad news from the FDA in January of this year as their drug Kyndrisa™ was rejected for unacceptable side effects but are continuing their application in the EU with the EMA. This has opened the door to a rival company Sarepta in the US for their application for the treatment of Duchenne Muscular Dystrophy (DMD) with their drug eteplirsen (Penumudi & Grove, 2016). The requirement to get an orphan drug to market before your competitor is an absolute necessity to establish a competitive edge. The rivalry BioMarin has experienced in the last few years is in its attraction as a takeover target (Garde, 2015) for larger biopharmaceutical companies who want to enter the orphan drug market or to reinforce their orphan drug market share with BioMarin's robust patented drugs and clinical trial pipeline.

Conclusion: Rivalry among competing firms would be considered high in the biopharmaceutical industry and BioMarin recent exploits with the FDA and Sarepta would support that conclusion. This recent issue with Kyndrisa™ is an exception to BioMarin's history. The type of rare conditions they treat and the limited patient pool that is associated with orphan drug designates does not attract high competitor rivalry and I would conclude that as a force it would be considered a **MEDIUM** threat to Biomarin.

(f) Relative power of unions, governments, special interest groups

Patient advocacy groups and rare disease societies have an influential power on the company due to the nature of BioMarin's drug portfolio treating rare and ultra-rare disease conditions. As a major contributor in the biopharmaceutical arena of orphan drugs and the development and commercialisation of drugs for diseases and medical conditions that previously had no treatment they are extending and improving the quality of life of their patients, significantly in children. Government agencies also exert influence on the external environment that BioMarin exists in. The recent issue in Europe with the death of a patient during the clinical trial of an experimental drug in France (Butler & Callaway, 2016) will only put further pressure on regulatory agencies who have the oversight to protect patients by not allowing new drug products into the patient domain until they are fully confident of their efficacy and safety.

BioMarin states that its workforce is non-unionised and that they have good working relationships with their employees and have had no interruptions to drug availability due to work stoppages as stated in their most recent 10K Report (BioMarin, 2016).

Conclusion: The influence and force that unions have on BioMarin are **LOW** due to good working relationships with employees. The force experienced due to special interest groups (e.g. Patient Advocacy Groups) and Government agencies (Regulatory Authorities) is considered **HIGH** with advocacy groups following the progress of possible new drug developments very closely due to the unmet need of a lot of rare diseases in the medical arena and government regulatory authorities overseeing the introduction of safe new treatments.

D. Summary of External factors

Table 2. EFAS Table (External Factor Analysis Summary) – BioMarin
(Wheelen & Hunger, 2008)

Opportunities	Weight	Rating	Weighted Score	Comments
R&D Tax Credits (Ireland)	0.15	2.0	0.30	Opportunity exists for tax credit on R&D spend in Ireland – No R&D activity in Cork facility
Corporate Tax Rate (Ireland)	0.05	3.0	0.15	No FDA approval of Cork facility for manufacture of Vimizim (testing & release approval only) – approval required to meet proposed commercial demand of Vimizim
Niche Market (Orphan Drugs)	0.20	4.0	0.80	Well positioned – current patented drugs on the market and solid R&D pipeline
Demographic Hubs (Education/Qualified & Competent Employees)	0.10	3.0	0.30	Well positioned in Biopharma international hubs
Sustainability (↓Water usage, ↑solar energy generation)	0.05	3.0	0.15	Well positioned with current & future plans
Threats				
FDA/EMA Regulations	0.15	3.5	0.525	Ongoing pressure from regulatory authorities will test strategies
Healthcare Budgets	0.10	2.5	0.25	Niche market – drug costs continually under pressure
R&D Costs	0.10	2.0	0.2	Extremely high R&D costs will test shareholders
Biosimilars (Generics)	0.05	4.0	0.2	Well positioned – future threat
IP & Patent Infringement	0.05	3.0	0.15	Well positioned at present – future threat
Total Scores	1.0		3.025	

IV Internal Environment; Strengths & Weaknesses

Table 3. IFAS Table (Internal Factor Analysis Summary) – BioMarin
(Wheelen & Hunger, 2008)

Strengths	Weight	Rating	Weighted Score	Comments
Commercialised Product Range	0.2	3.5	0.7	Five products with market monopoly
Capacity	0.1	4.0	0.4	California expanded & Shanbally
Partnerships	0.1	3.5	0.35	Genzyme, Catalyst
Distribution / Infrastructure	0.05	2.5	0.125	USA, Latin America, Asia, Europe
Skilled Workforce	0.1	4.0	0.4	BioMarin & Acquisitions
Weaknesses				
Pipeline Failures	0.2	3.0	0.6	New product must replace old
Niche/Small Market Populations	0.125	4.0	0.5	Reduced revenue potential = high prices
R&D Costs – major strain on profits	0.125	2.0	0.25	Notable high R&D Costs
	1.0		3.325	

V Analysis of Strategic Factors

BioMarin Pharmaceutical Inc. (BMRN) is a global biotechnology company who specialises in the development and commercialisation of cutting edge biopharmaceuticals for treatment of rare & ultra-rare genetic diseases. These patients make up the minority of the global population. The company's operating headquarters is out of San Rafael, California and employs 2000 people as of the 12th April 2016. The company has a diverse geographical span in that they've facilities in the United States of America, Europe, South America & Asia. Records show the company earned \$889,895 million during fiscal year ending December 31st 2015, an increase of 18.8% over fiscal year 2014. The operating income of the company stood at \$110.7 million in 2015 compared to an operating income of \$92.9 million. Net income amounted to \$171.8 million in contrast to net income of \$134 million in 2014.

Table 4. SFAS Table (Strategic Factor Analysis Summary) – BioMarin

(Wheelen & Hunger, 2008)

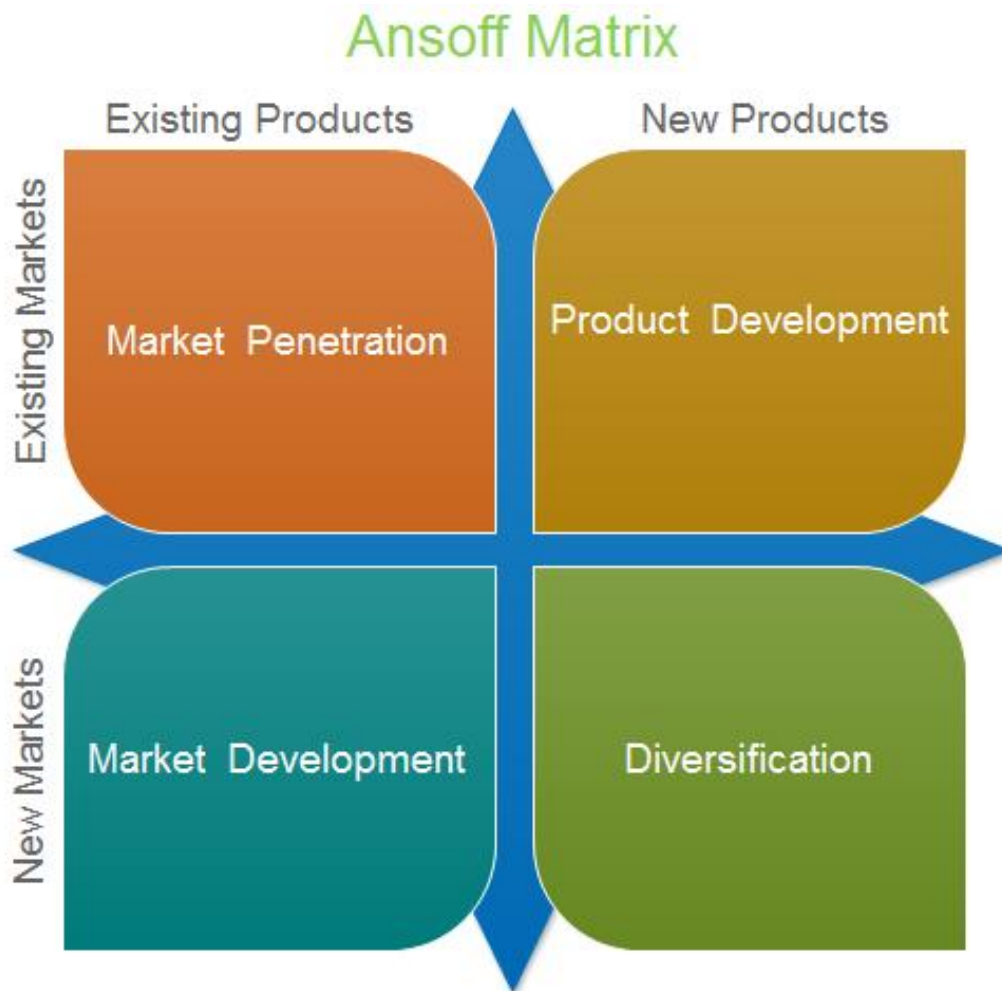
Key Strategic Factors	Weight	Rating	Weighted Score	Short	Intermediate	Long
Commercialised Product Range	0.2	4	0.8			X
Capacity	0.05	3.5	0.175		X	
Skilled Workforce	0.1	3.5	0.35		X	
R&D Tax Credits	0.15	4	0.6	X		
Niche/Small Markets Populations	0.15	3.5	0.525			X
Corporate Tax Rate	0.15	3	0.45		X	
FDA/EMA Regulations	0.05	4	0.2			X
Shareholder Pessimism – High R&D Costs	0.1	3	0.3		X	
High R&D Costs	0.05	3	0.15			X
	1.0		3.55			

Key Facts

Headquarters:	San Rafael, California
Founded:	1997
Chairman and CEO: Employees:	Jean-Jacques Bienaimé 2000
Ticker:	NASDAQ: BMRN
Revenue 2015:	\$890 Million
Net Income (Loss) 2015:	(\$171.8 million)
Focus:	Rare and ultra-rare genetic diseases
Products:	Vimizim® (elosulfase alfa) for (MPS IVA) Naglazyme® (galsulfase) for MPS VI Aldurazyme® (laronidase) for MPS I Firdapse® (amifampridine phosphate) (currently approved in the EU only) for LEMS Kuvan® (sapropterin dihydrochloride) Tablets for Oral Use and Powder for Oral Solution for PKU
Clinical Pipeline:	<u>Pegvaliase (Peg-Pal): for PKU</u> <u>Reveglucosidase Alfa (BMN 701): GILT GAA for Pompe Disease</u> <u>Vosoritide (BMN 111) Analog of CNP: for Achondroplasia</u> <u>Cerliponase Alfa: for CLN2 disease</u> <u>BMN 270: AAV-Factor VIII Vector for Hemophilia A</u> <u>BMN 250: GILT rhNAGLU for Sanfilippo Syndrome/MPS IIIB</u> <u>Drisapersen: for Duchenne Muscular Dystrophy (exon 51)</u> <u>BMN 044: for Duchenne Muscular Dystrophy (exon 44)</u> <u>BMN 045: for Duchenne Muscular Dystrophy (exon 45)</u> <u>BMN 053: for Duchenne Muscular Dystrophy (exon 53)</u>

Partners:	Genzyme Corporation Merck Serono Catalyst Pharmaceutical
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BioMarin Pharmaceuticals Inc. are pioneers in sourcing, developing & commercialising therapies which plagued minorities domestically & internationally. “We aim to bring new treatments to market that will make a big impact on small patient populations.” Using cutting edge enzyme replacement therapies (ERTs), technology, research, knowledge accrued over the past 19 years BioMarin has made dramatic impacts on its patients lives. Once again Naglazyme came out on top as the main revenue earner. Naglazyme is used for medical assistance of mucopolysaccharidosis VI or MPS VI. It contributed the highest percentile of 34% of the total revenue earned for the company. Vimizim had a very profitable year in sales jumping up by 195% from 2014. Vimizim used for the medical care of mucopolysaccharidosis IV Type A, or MPS IV A. Vimizim as a % of total revenues was 25.6% in 2015 compared to 10.32% in 2014. Kuvan also had a strong year for the company with an increase 17.9% over 2014. Kuvan as a % of total revenue was 26.9%.



(www.cacmaccountants.ie, 2016)

From examination of the combined EFAS & IFAS tables into an SFAS table the highest weighted score achieved is for The **Current Commercialised Product Range** scoring 0.8. This does correlate to the company's objectives and strategies of Market Penetration.

Commonly referred to as the “Protect & Build” strategy, a conservative outlook or perspective will be adopted by the company to lay foundations for dominance of particular sector of the market & stabilise position by selling more existing products to existing customers. Factoring in this cross selling approach, for this to run efficiently & effectively the company needs to leverage existing resources & capabilities which in turn will lead to attainment, capture & securement of larger share of the market. The low risk associated to the strategy means no need to launch new product or service immediately, instead the focus will be directed at selling more products to existing clients & contacts. BioMarin anticipated the demands to increase from financial forecasting (Vimizim, Kuvan) so they were waiting to be successfully given permission to put plans in play.

R&D Tax Credits achieved a weighted score of 0.6 and in the overall scheme of things has short effect on the company's earnings. BioMarin has been granted approval from FDA/EMA for testing & release of Vimizim & have begun manufacturing Vimizim. Previously it was only manufactured at the Novato facility in California. This will allow Vimizim to be commercialised to meet demands in Europe & Rest of the World & takes pressure & responsibility of it all being concentrated at the Novato facility. It does satisfy the plans in play BioMarin had implemented for Market Development when they purchased the Shanbally plant from Pfizer in 2011. They anticipated sales to rise after securing approval from FDA in 2014 to market & distribute Vimizim so they were waiting to be granted quality approval to manufacture from the plant in Cork. The benefit of R&D tax credit of 25% & corporation tax of 12.5% is added incentive. (KPMG, 2016).

Niche/Small market populations is the next strategic factor up for review. Falls into the strategy brackets of Product Development/Focused Diversification. Product Development this strategy ultimately defined as focusing & concentrating on creating new products & presenting them to existing customers. BioMarin is continuously striving to bring new innovations, new drugs which are in clinical development to market as soon as they've secured licenses to do so. The key to success is monitorisation & utilisation of market research in order to recognise, identify, determine & distinguish gap in the market for new product(s). Marketing approval for orphan drug status can have an easier route to market because it's very specialised niche which focuses on smaller populations, but financially very rewarding as competition more confined (Hadjivasilion, 2015) (Almashat & Sorscher, 2015). The financial incentive such as extended exclusivity allows time for preparation & upon obtaining market approval you can dominate the market controlling price of drug. Medical breakthroughs which may have been overlooked due to lack of demand & cost invested in drug research & development is a matter of public policy in many countries (Armstrong, 2010). That's where BioMarin has tapped into virgin territory. Focused Diversification, if plausible evidence identified that BioMarin possesses particular drug which meets high enough demand or a sector of the market has been probed which shows there's a likelihood of success such as Talazoparib a *PARP inhibitor shown clinical activity against cancers (ovarian & cervical)* ended up being sold to Medivation for \$570million BioMarin (2016) K-10 because it didn't fall in line with orphan drug status. *Mr. Fuchs and BioMarin's overall strategy of commercialising Orphan drugs only.* (Pegvalaise-(PKU), Reveglucosaide-Pompe disease, Cerliponase alfa-late infantile Batten disease & BMN-044,045,053 & Drisapersen all for *Duchenne Muscular Dystrophy*.) The result of success far outweighs the means of processing & application. The risk element is inherent with this strategy as developing new products requires investment from the business.

Skilled Workforce scored 0.35 from SFAS table. Having a skilled workforce at your disposal is integral for the company to have longevity. The company needs competent workforce to produce, manufacture, research & develop these products for market & to assist patients needs.

To operate, maintain & sustain a biotechnology company which will possess longevity the business model it adapts must secure financing first of all. The second thing the company needs to do is manage to occupy share of the market with either new commodity or improvement on existing commodity (securing legal rights to do so) & make use of its operational strategies. What business strategy the company adopts will all depend on availability of collateral & willingness of investors to invest in what BioMarin are offering. BioMarin has made use of its resources to adapt in a competitive market intersecting & taking advantage of these 3 business models on which tactical and strategic plans can be executed by the company:

- Build: **Current Commercialised Product Range** & advancing pipeline drugs.
- License: **Niche/Small market populations** BioMarin has exercised dominance in the biotechnology industry through acquisitions, alliances & collaborations within niche orphan drug market. Procurement of intellectual property from acquisition, alliance, partner, or collaborators enhances strength & dominance within the market. Expanded grasp geographically by operating globally. Demographic hubs in North America, Latin America, Australasia & Europe.
- Sell: Having manufacturing & distributing channels in play allows for expansion into new markets & market development. **R&D Tax Credits** of 25% & 12.5% are an alluring prospect of attracting BioMarin to Ireland (Tsaa & Erickson, 2006) (KPMG, 2016).

VI. Strategic Alternatives

Business strategy

Cost competing strategies are not a significant consideration for Biomarin at this point in time. They have little or no competitors in their current niche market portfolio. All five of their commercialised drugs have or previously had orphan drug status and three of them have entered the market as the only treatments available for the rare disease it treats.

Biomarin could adopt an alternative broader **differentiation** strategy but this would entail broadening their product portfolio to include more generic products.

They have dipped their toes with this strategy when they acquired a company called Lead Therapeutics in 2009 to gain proprietary rights over a breast cancer drug, Talizorib, spending \$181.6 million to get it to phase three clinical trials before selling it to Medivation for \$570million in 2015, realising a net gain of \$369.5million.

Not stated as to why Biomarin sold this drug when it held strong commercialisation opportunity, but it was the only drug in the Biomarin portfolio that did not have orphan drug designation.

The most likely reason for this tactic was not to encroach upon the market share of larger companies, become their competitor and make them a target for acquisition.

Moving into markets with higher patient populations could potentially present a significant culture change for the workforce. Sometimes meeting the patients they treat, scientists are strongly motivated in their work which can have a direct impact on the likelihood of survival for a patient. In contrast, developing drugs where treatments already exist do not necessarily induce the same level of motivation.

Of Porters four generic competitive strategies, Biomarin can be described as following the **focussed differentiation** strategy for its business. It is differentiated because their products are developed to treat rare and ultra-rare diseases, with all five of their commercialised drugs having had or have orphan drug status.

It is focussed because the products are sold to a very niche market. It is not targeting the market centre, therapeutics in general, but a specific niche market away from centre.

Developing drugs that are granted orphan status makes the company a first mover, allowing it to establish itself as a market leader and offer further improved products thereby dominating the market. The following tactics are used to achieve this focussed differentiation strategy.

It has a laser focus on the FDA approvals process and the 1983 Orphan Drug Act (ODA), which helps to ensure orphan drug status designation.

To reap the benefits of these designations, it hires strong accounting expertise to take advantage of the tax incentives offered. Tax credits of 50% are granted for clinical trials.

Mainstream new drug applications normally take ten months to review, whereas orphan drug applications have priority review and completed within six months. *Smart-therapeutics.com*. (2016).

Tax credits can also be carried forward from year to year, for up to 20 years depending upon the incentive applied for. Rarediseases.org. (2016).

The company has recruited to the board of directors, some of the most highly recognised expertise in their field, thus ensuring they receive the most up to date information and good advice in regards likely future acquisitions, partnerships and drug portfolio options.

Functional strategy

As a **functional** strategy Biomarin create opportunity by training doctors in how to diagnose patients for diseases treated by their drugs. This program yields a number of benefits whereby Biomarin can build close relationships with doctors and provide them with free expertise. This not only generates market opportunity, but ultimately leads to a pull system, whereby doctors can quickly diagnose the rare condition and prescribe the Biomarin drug.

If the disease is rare and a doctor does not have the pre-requisite training, the disease may not be diagnosed quickly, it may be diagnosed at a later stage or not at all.

Another example of functional strategies employed at Biomarin is its 50/50 partnership with Genzyme, whereby Biomarin produce the drug Aldurazyme, whilst Genzyme market, distribute and sell it.

Corporate strategy

A **stabilising** strategy is to pause and stop growing which is highly inadvisable for Biomarin in a growing industry. This would focus efforts on their existing commercialised products and pipeline drugs, curtailing expenditure into new drug acquisitions and partnerships.

The strategy could make sense for a short period of time, to balance the books and allow more borrowing, but once market exclusivity is lost through the IP/patent cliff, the possibility of competition can quickly decimate revenues leaving the company in a very vulnerable position.

Retrenchment requires a company to turn itself around from a weak position in its market and concentrate on its operational in-efficiencies. This not a requirement for Biomarin as they are currently enjoying a vigorous growth phase.

Growth Strategy

Biomarin is following an aggressive growth strategy which is very suited to achieving their current objective of sourcing, developing and commercialising therapies for rare and ultra-rare diseases. This conclusion is based on the following criteria.

The market capitalization of the company has grown from \$450million to \$15billion in ten years, increasing the company in size 33 times from 2005 to 2015

Aldurazyme, the organisations first commercial drug, launched in 2003, earned the company \$76.4million in net revenue for 2005. The orphan drug status of this product expired in 2010 but the drug is still generating revenues of \$98million and \$105.6million for 2014 & 2015 respectively.

The ability of Biomarin to analyse their niche market and choice of diseases to target, demonstrates their ability to choose drugs that can deliver orphan drug status and generate enough revenue to cover R&D and operational costs. Their superior R&D and first to market strategy helps protect it even after market exclusivity has lapsed.

The product portfolio has grown from one commercial product in 2003 to five commercialised products in 2015 earing net revenues of \$885.5million.

It has formed at least nine partnerships since 1998 and in the past seven years it has acquired five companies and bought the rights to a drug from Repligen.

To accommodate its growth in sales for Vimizim, it expanded production facilities in Novato California and purchased the Shanbally production facility in Cork.

The company released a product almost every two years up until 2010 and four years later it released its blockbuster drug, Vimizim in 2014. See Fig 1.0 below showing release and orphan drug expiry dates.

Date approved US	Date to expire US	Date approved Europe	Date to expire Europe
2014	2021	2014	2024
2007	2014	2008	2020
2005	2012	2006	2016
2003	2010	2003	2013
Unapproved in US	N/A	2010	2020

Fig 1.0

Recommended Strategy

This TOWS table was developed to help generate alternative strategies that could be used to strengthen and defend their market position. See Fig 2.0 below

Fig 2.0

TOWS Table	Strengths	Weaknesses
Internal factors (IFAS)	Commercialised product range	Pipeline failure
	Capacity	Niche/Small market populations
	Partnertships	R&D major strain on profits
	Distribution/Infrastructure	
	Skilled Workforce	
External factors (EFAS)		
Opportunities	1) Transfer commerical products to a lower corporate tax area 2) Look for areas to expand market. 3) Partner with companies in areas with lower corporate tax? 4) Expand infrastructure to areas of further market potential. 5) Manufacture comercialised product close to	1) Put significant resources into disease product selection to ensure FDA orphan drug status. 2) Develop products in countries outside US? 3) Examine Irelands 5% R&D tax incentive program. 4) Work with external organisations to develop pipeline examples Universities & NIBRT 5) Ensure the full orphan drug tax credit (ODTC)
R&D Tax Credits		
Corporate Tax Rate		
Niche Market (Orphan)		
Demographic Hubs/Surrounding resources		
Sustainability	1) Ensure full GMP/GDP compliance is met with the existing commercial products range to ensure uninterrupted revenue. 2) Scrutinise product costs with a view to understanding if it would be more profitable to charge less & sell more? Understand if health provider and insurance companies are not precriding their drugs based on costs. 3) Publish to shareholders the benefits of purhcasing stock in their company e.g. guranteed revenue from orphan products. 4) Defend position against biosimilars by holding back IP as much a possible. 5) Monitor potntial IP infringement. Small change, large effect. Bullwhip effect. 6) Mnitior comapnies developing products or technology that could impat revenues from commerclaised or pipeline products - Board of directors. Assess acquisition. 7) Ensure new facilities are planned years into the future, guaraneteeing capacity for existing product, but also new products in pipeline.	1) Laser focus on regulations. 2) Focus on R&D costs to lower the price of a product when it goes to market. Innovative R&D. 3) Improve upon existing product to defnd market position i.e. canniblise own product like the Intel strategy. (Biomarin are already doing this.) 4) Pursue more benefits from regulations. 5) Develop relationship with healthcare providers & insurance groups to work together in regards pricing. 6) Laser focus on product dvelopment and production costs versus what the market is willing to pay? 7) Publish potential rewards to shareholders. 8) Evaluate costs to upgrade existing product to defnd market share. 9) Possible to defend IP at design stage?
Threats		
FDA/EMA Reg		
Healthcare Budgets		
Shareholder pessimism - High R&D costs		
Biosimilars		
IP/Patent Infringement		

It is highly recommended for Biomarin to follow its current growth strategy.

The following recommendations should be considered, if not already being acted upon.

Keep a laser focus on its cash flow, R&D expenditure, acquisition and operational costs.

The company may want to consider a degree of stability at some point in the near future considering the 2015 annual report states the company has high indebtedness to the tune of \$781.4 million.

Investors.bmrn.com. (2016). *BioMarin Pharmaceutical Inc. - Annual Report*.

In 2015 alone it acquired Prosensa \$538.4million, operating expenses \$221.7million, \$227.7million in plant and equipment, \$371.8million for global rights to Kuvan and the purchase of \$327.8million in long term investments.

The FDA/EMA regulations need to be rigorously monitored for opportunities and to defend against threatening amendments.

The 1983 Orphan drug act needs to be scrutinised and defended where possible as this act is very much a reason why the company exists in the first instance as it provides up to 50% of the costs for clinical trials.

Rarediseases.org. (2016).

Developing drugs for rare diseases can be attractive because they offer priority review from the FDA for submissions and approvals. The FDA facilitates this as part of the Orphan drug act, because patients, in many cases, do not have any other alternative treatments.

The priority review greatly accelerates the time to market, normally taking ten months to review a new drug application (NDA), with priority review it takes just six, along with seven years market exclusivity in North America, *Fda.gov*. (2016). *Orphan Drug Act.*, and ten years market exclusivity in Europe, *Ema.europa.eu*. (2016).

Biomarin recently sold a priority review voucher to Regeneron for \$67.5million.

It was of no value to them because they receive priority review anyway.

The Orphan Drug Act (ODA) provides a structural barrier which protects Biomarin in the commercialised market, but it does not protect them in the upstream process.

With the increase of companies coming into the biotechnology market, it reduces the resources available to Biomarin, as other companies may beat them to the mark when it comes to acquiring companies, forming partnerships and strategic alliances.

They defend against this threat by recruiting highly experienced personnel to their board and top management team, thus keeping themselves abreast of potential opportunities.

A core competency at Biomarin is their expertise in enzyme replacement therapy (ERT).

Four from five of their commercialised drugs are based on this technology. Naglazyme is the only drug in existence for MPS VI. Kuvan was the first treatment drug for PKU. Aldurazyme, for the treatment of MPS I, is the only treatment of its kind that exists. And Vimizim is the only ERT treatment available for MPS IV A.

Applying Barney's VRIO framework, each of these products would answer 'yes' to each principle of Value, Rareness, Imitability and Organisation. It would make sense to continue leveraging growth from this technology.

Capitalise on tax credit carryforwards (\$143,987million) and net operating loss carryforwards (\$44,942million).

Avoiding threats is a key part to the Biomarin strategy. The examples below demonstrate this and should continue to be used to help strengthen their market position.

If the Orphan Drug Act 1983 (ODA) were to be repealed, the Biomarin operations and long term future would be in potential jeopardy. The Biotechnology Industry Organization and the National Organization for Rare Disorders protects the orphan drug act where possible and produced a report in 2015 where it is concluded that without the ODA, up to 33% of rare disease drugs would not make it to market over the coming ten years. *Rarediseases.org*. (2016). Biomarin is an active member of this organisation and defends its position through reports such as these.

If Biomarin is granted orphan drug status, no other drug company will be granted an orphan drug licence and the only circumstance under which this will change is if Biomarin cannot produce enough quantities of the drug or there is sufficient evidence an alternative drug is superior to the Biomarin equivalent.

This regulation helps reduce the threat of competition.

Talizorib did not fit with their orphan drug portfolio and selling it was most likely a tactic to keep the bigger drug companies at bay and stay off their acquisition radar. This demonstrates innovation, flexibility and threat avoidance.

Biomarin recognise the threat of raw material sole sourcing. As stated in the annual K-10 report, *Investors.bmrn.com*. (2016). *BioMarin Pharmaceutical Inc. - Annual Report.*, they have implemented multiple sourcing strategies for critical raw materials. They have five products and a delay or loss of supply, for a critical raw material, could have disastrous consequences for the company. It would be worth investigating the acquisition of such a supplier, thus implementing a vertical integration strategy.

If this company were to choose any other strategy in the niche market they operate within, the company would be putting itself into a vulnerable position. It is recommended to pursue their current growth strategy through in house product development, partnerships, acquisitions and alliances.

VII. IMPLEMENTATION

BioMarin is a biopharmaceutical company which develops, manufactures and distributes drug products for the treatment of rare and ultra-rare medical conditions. The company's strategy is based on discovering and/or the acquisition of a drug product or potential drug product that can be developed to treat an unmet medical need and to gain orphan drug designation for that product (orphan drug designation <200,000 US patient population), (www.fda.gov, 2013).

BioMarin's current drug portfolio consists of patented orphan status drug products and a pipeline that has drugs at all stages of pre-clinical and clinical trial. On paper this is surely where the company wants to be to achieve its strategy goals but the recent unfavourable review of its Duchenne Muscular Dystrophy (DMD) drug Drisapersen® highlights the financial risks involved in BioMarin's strategy when one considers that BioMarin purchased Drisapersen for potentially US\$680 million from Prosensa in 2014 (Carroll, 2014). This is the risk when operating a differentiated business strategy which requires innovation and taking its opportunities in an extremely competitive industry (Greiner, 1998) with the requirement to be first to market in a niche segment of the pharmaceutical industry. BioMarin's future acquisitions of intellectual property are integral to its future success and will depend highly on the people they have investigating potential future opportunities. At a corporate level, directors must ensure that these people have the resources they require to achieve the required milestones for the company in appropriate and financially viable new acquisitions.

With the company presently suffering from year on year financial losses on its balance sheet and patent expiries looming, the introduction of programs to consider production and other operational efficiencies will need to be considered. The introduction of lean six sigma as a corporate program, would set corporate, department and employee goals which when aligned, will allow all employees from senior management down, to analyse their roles and responsibilities and where they can cut costs or add value to their jobs and workplaces. The program should be ran from a corporate level but one which should be owned by every employee.

The acquisition of the Shanbally facility in Cork has increased BioMarin's manufacturing capacity and the FDA approval of the facility for the manufacture and commercialisation of Vizimum® is a significant milestone that needs to be achieved in the immediate future to secure financial return on its facility investment. The financial return on Vimizim® production at Shanbally is of further incentive when considering Ireland's corporate tax rate.

Shanbally and BioMarin's Dublin commercial hub offer significant opportunities for BioMarin's distribution networks with the potential for European manufactured products supplying the European markets. This will simplify the network and if aligned and operated in synergy with the rest of the network will aid quick supply to the market.

Sustainability is an important corporate feature of BioMarin's to date operations. Implementation of current and future sustainability policies and projects will require co-ordination as BioMarin's employee numbers and areas of operation increase. Corporate governance boards must ensure that all employees in all locations are equipped with training and opportunities to participate in all areas of sustainability and encourage new sustainability projects which are specific to their location or environment.

The challenge for BioMarin, as it sets up strategically important manufacturing and business operations on this side of the Atlantic, is to maintain open lines of communication among all departments of the company and for co-ordination of all internal policies and programs as employee numbers increase worldwide.

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Appendices

Appendix 1

Director	Current Roles	Previous roles
Elaine J. Heron	Biomarin-Director Amplix-Director (Chair) Zephyrus Biosciences-Director	Biotech-Executive Amplix-CEO, 2009-2012 Labcyte-CEO & Chair, 2001-2008 Applera-General Manager & Vice President Sales, 1996-2001
Pierre Lapalme	Biomarin-Director Aeterna Zentaris-Director Insys Therapeutics-Director Pediapharm Inc-Director	Ethypharm President & CEO, 1995-2003 Rhône-Poulenc CEO North America Ethicals, A branch of Rhône-Poulenc-Senior Vice President & General manger Bioxel-Director, 2004 – 2009
Michael Grey	Lumena-President & CEO (Acquired by Shire for \$260million) Amplix-CEO & President Reneo-Founder, CEO, Chair Biomarin-Director Biothera-Director Horizon-Director Mirati-Director Ziarco-Director Pappas Ventures-Director Balance Therapeutics-Director Selventa-Director	Auspex-President-CEO, 2009 SGX-President-CEO, 2005-2008 (Acquired by Eli Lilly) SGX-President, 2003-2005 SGX-Chief Business Officer, 2001-2003 Trega Biosciences-President-CEO, 1998-2001 BioChem Therapeutics-President, 1994-1998 Ansan-President & COO, 1994 Glaxo Holdings-Vice president & various roles, 1974-1993 IDM Pharma-Director, 1999-2009 Achillion-Director, 2001-2010
Jean-Jacques Bienaime	Biomarin-Chair & CEO Incyte Corporation Vital Therapies Biotechnology Industry ORG	Genencor-CEO&President&Chair,2002-2005 (Sold to Danisco) Sangstat Medical- CEO/President, 1998-2002 (Acquired by Genzyme) Rhône-Poulenc Rorer,1992 to 1998 (Acquired by Sanofi-Aventis) Worked at Genentech earlier in his career
Alan J. Lewis	Cellastra-Chairman DiaVacs-Director Targazyme-Director	Medistem, 2012-2014 Ambit Biosciences - CEO Director, 2010-2011 Diabetes Foundation - President CEO, 2009-2010 Novocell - President CEO, 2006-2008 Celgene - Signal Research President, 2000-2006 Signal pharmaceuticals President, 1994-2000 (Acquired by Celgene 2000) Wyeth-Ayerst - Research Various positions, 1979-1995

Appendix 2

Director

Randy Meier

Current Roles

Staar Surgical, Director

Owens & Minor Inc, *President-
International, Executive Vice
President & Chief Financial Officer*

Management & Financial advisor

V. Bryan Lawlis

Itero llc, President & CEO
Itero llc, Director
Sutro Inc, Director
Geron, Director
KaloBios, Director
Coherus, Chairman Scientific
advisory board

Dennis J.
Slamon, M.D.,
Ph.D.

Revlon/UCLA Women's Cancer
Research Program, Director

Hematology/Oncology Division of
UCLA, Chief & Executive Vice Chair

David Pyott

National Colorectal Cancer
Research Alliance, Director of the
medical advisory board.
Avery Dennison, Director
Alnylam, Director
Royal Philips, Supervisory Board
Member
Chapman University, Vice
Chairman of the board of Trustees
London Business School, member
of the Governing Board.
International Council of
Ophthalmology Foundation,
President
Foundation of the American
Academy of Ophthalmology,
Member of the advisory board.

Previous roles

Teleflex - Ex Vice President & CFO, 2010-2012
Advanced Medical Optics - President & COO, 2007-2009
(Acquired by Abbott in 2009)
Advanced Medical Optics - CFO, 2002-2007
Valeant - Exec Vice President CFO, 1999-2002
Senior Vice President Treasurer, 1998-1999
Schroder & Coin, 1996-1998
Other banking positions: Salomon Smith Barney, Hanover
Corp, Australian Capital Equity, Greyhound Lines Inc
ITero Inc. - Founder, President & CEO, 2006-2011
Paradigm - President & CEO, 2004-2006
Paradigm - President & COO, 2003-2004
Paradigm - COO, 2001-2003
Covance - Co-Founded, President & CEO, 1996-1999
Covance - Chairman until sold to Diosynth, 1999-2001
Genencor & Genentech, last position Vice President of Process
Sciences, 1981-1996

Allergan - CEO, 1998-2015 (Acquired by Actavis)
Novartis - Head of Nutrition, 1995-1997
Sandoz Nutrition - President & CEO, 1992-1995
Sandoz Nutrition Spain - General Manager, 1990-1992
Sandoz Nutrition, 1980-1990

Appendix 3

Director

Elaine J. Heron
Pierre Lapalme

Background

Academic
Rolled out ethical programs in the States, Canada, Mexico, Central America and had a role on the board of the Pharmaceutical Manufacturers Association of Canada.
Strong experience with patents and heavily involved in re-instituting patent protection for pharmaceuticals

Skills/Experience

Biosystems & chemistry
Drug Delivery

Michael Grey

Founded Reneo
Founded Lumena – Shire Acquisition
President & CEO SGX – Eli Lilly Sold.
President & CEO Trega Biosciences – Lion Bioscience Acquisition

Founded a number of companies. Fungal infections

Jean-Jacques
Bienaime

Grew Biomarin from a \$450million market capitalization company to a \$15billion market capitalization company in 10 years.

Economics & Business.
Strong Biotechnology & Pharmaceutical experience having worked with Genencor who develop products targeting cancer, and developed it for sale to Danisco for 1.2 billion. Worked with Rhone Poulenc Rorer who also developed drugs for lung and breast cancer.

Dr.Alan J. Lewis

Academic – Research 15 years.
President & CEO of Signal- Celgene. Sold

Varied education across pharmacology and biochemistry. Spent 15 years researching for Wyeth and then transferred into more mainstream management. Strong experience in biotechnology, but also across stem cell therapy, regenerative disease biotechnology, pharmaceuticals and diabetes.

Randy Meier

International experience and varied.
CEO of Advanced Medical optics before it was acquired by Abbott.

Finance/Economics

V. Bryan Lawlis

Academic
Founded Covance a biotech manufacturing company that sold to Diosynth.

His career has generally been centred in biotechnology production having founded a company providing biotechnology contract services and also held a position with Genentech as Vice President of Process Sciences. He brings great experience to the Biomarin for Biotechnology production.

Appendix 4

Director

Dennis J. Slamon,
M.D., Ph.D.

Background

Academic – 20 Years research
Introduced Herceptin.

Spent the past 20 years researching cancer.
Developed Herceptin, which extends the
life of women with breast cancer.
His work has changed the way cancer is
treated and given many awards for his
work on cancer, especially breast cancer.

Skills/Experience

Mr Slamon has been a researcher all his life and
received numerous awards for work into cancer
research and developed a breakthrough drug
that extends the life of up to 25% of women
suffering from breast cancer.
He brings enormous experience in regards
cancer research. Having recently joined the
board at Biomarin, it must be asked if Biomarin
are looking into making drugs to combat cancer.

David Pyott

One of the top 25 highest paid CEO's in the
United States.
Increased revenue of Allergan from
\$1Billion to \$7billion in 17 years.
One of the longest tenured CEO's in the
pharmaceutical industry.
International experience, with Sandoz and
Novartis.

Business acumen.

Appendix 5

Manager

Robert A.Baffi

Current Roles

KaloBios - Director

National Institute for
Bioprocessing Research &
Training - Director

Previous roles

Genentech – Quality
Cooper BioMedical - Research
Becton Dickinson - Research

Joshua A.Grass

Biomarin - Senior Vice President,
Business & Corporate
Development

Vrolyk & Company – Associate, Mergers Acquisition.
BioMedical Insights – Research Analyst
SG Cowen- Research
Cerus – Research & development cell biology

G.Eric Davis

Biomarin - Executive Vice
President and General Counsel

Paul Hastings Janofsky & Walker LLP

Jeff Ajer

Biomarin - Executive Vice
President and Chief Commercial
Officer

Genzyme
SangStat Medical Corporation
ICN Pharmaceuticals

Scott Clarke

Bio Marin - Senior Vice President
Product Development

PhotoBioChem BV
Cerus Corporation

Philip Lo Scalzo

Biomarin - Senior Vice President,
Chief Compliance Officer

Sanofi-Aventis
Sedgwick, Detert, Moran & Arnold - Attorney

Eduardo E. Von
Pervieux

Biomarin - Vice President
Information Technology, Chief
Information Officer

Amgen
Pfizer

Dan Spiegelman

Biomarin - Executive Vice
President and Chief Financial
Officer

CV Therapeutics
Genentech

Relpysa Inc-Chair
Oncothyreon Inc-Director

Richard Ranieri

Biomarin - Executive Vice
President, Human Resources and
Corporate Affairs

Dendreon Corporation
Sepracor, Inc
Neurocrine Biosciences, Inc
Genencor International
Smithkline Beecham

Dr. Henry J. Fuchs

Biomarin - Executive Vice
President and Chief Medical
Officer

Onyx Pharmaceuticals
Ardea Biosciences
Genentech

Mr.Brian R. Mueller

Biomarin - Group Vice President,
Corporate Controller and Chief
Accounting Officer
Anthera - Director

Arthur Andersen LLP
KPMG LLP

Appendix 6

Manager	Background	Skills/Experience	Education Background
Jean Jacques Bienaime Robert A.Baffi	Leadership Quality	Management. Oversees manufacturing, process development, quality & analytical chemistry. Took part in 20 product approval submissions to the regulators in Europe and the US. Took part in more than 50 regulatory submissions for new drug investigational testing.	Genentech, Genencor, Rhone-Poluenc Rorer Ph.D. MPhil, & B.S. Biochemistry
Joshua A.Grass	Financial Relations	Raised \$1billion in capital.	B.S. Biology. M.B.A. Rochester University New York
G. Eric Davis	Legal		B.A. Political Economy – Berkeley J.D. - San Francisco school of law
Jeff Ajer	Marketing	One of the first Marketing/Sales employees	B.S. in Chemistry. M.B.A., University of California, Irvine.
Scott Clarke	Product Development	Chooses product pipeline Alliance formations etc. Led development in drug device combination.	M.B.A London Business School B.S. Chemical Engineering Berkeley B.A. Biotechnology Northwestern Uni
Philip Lo Scalzo	Compliance	Audits, Ethics, Corrective Actions	J.D. from Brooklyn Law School B.A. in History from Boston College.
Eduardo E. Von Pervieux	IT	Implemented Global IT system for supply chain technologies. Extensive experience working internationally.	M.B.A and M.I.B. from the University of Miami B.A. in economics
Dan Spiegelman	Chief Financial Officer		B.A. from Stanford University. M.B.A. from the Stanford Graduate School of Business

Appendix 7

Manager	Background	Skills/Experience	Education Background
Richard Ranieri	Human Resources		B.A. from Villanova University M.A. Organizational development, Rider University.
Dr. Henry J. Fuchs	Medical Officer	Led program that approved Pulmozyme Phase 3 development program that led to the approval of Herceptin to treat metastatic breast cancer	M.D. degree George Washington University B.A. degree in biochemical sciences, Harvard University.
Mr. Brian R. Mueller	Accountant	Chief accounting officer KPMG – Audit & Transaction advisory services.	B.S. in Accountancy, Northern Illinois University