VOLT® PHARMA ASSOCIATES

Is Orphan Drug Status the Answer to Everyone's Prayers?

Orphan drug development is a risky venture. The small number of patients, despite the premium price, may not lead to huge revenues and there is a significant risk of failure to reach Proof of Concept. There is no doubt that orphan drug development is highly suited and can be extremely profitable to smaller companies and larger ones looking to expand their product portfolios. While the costs and time needed to get to registration may be substantially smaller, there are many risks that can be mitigated by a clear understanding of the process and changing environment.

Table of contents

- Executive Summary
- Introduction
- Why Do Small Drug Companies Find It Attractive To Develop Orphan Drugs?
- Why are Big Pharmaceutical Companies Developing Orphan Drugs?
- Is Orphan Drug Development Economically Viable?
- What Needs to be Optimised in Orphan Drug Trials?
- Possible Future Challenges in Orphan Drug Development
- References

Website

www.vpa.eu.com

Executive Summary

There are an estimated 7,000 rare diseases affecting approximately 350 million people globally. Eighty percent of these are of genetic origin and approximately fifty percent of those affected are children. The conditions are mostly chronic, degenerative or life-threatening and in serious need of treatment. Recent advances in the understanding of molecular processes and genetic testing have led to a better understanding of the underlying science and enabled viable approaches to be identified. The development of drugs for these rare conditions can provide significant opportunities for both large and small pharmaceutical companies.

For large companies the advantages include: there may be research tax credits, funding grants and waivers of regulatory fees; clinical trials are smaller and cheaper; patients and physicians treating the conditions are easy to find; new effective drugs can achieve premium pricing; because of the small numbers marketing costs are reduced; gross profit margins are massively higher than for drugs for more common conditions and there may be a longer period of exclusivity.

For small companies the advantages include: no need to build a development organisation; significant commitment and assistance is available from the regulatory authorities who are also willing to commit to agreements on the requirements for registration; the size of the clinical trials and, therefore, the development time and costs are a small fraction of that required for a 'blockbuster'' and the potential for a rapid progression to market makes an orphan drug development an attractive option for a venture capital company as it means a possibility of an early exit with significant return on their investment.

Orphan drug development is still a risky venture. The small number of patients, despite the premium price, may not lead to high revenues and there is a significant risk of failure to reach Proof of Concept. And once this has been achieved, the probability of getting to market is likely to be much the same as the development of a 'normal' drug treatment.

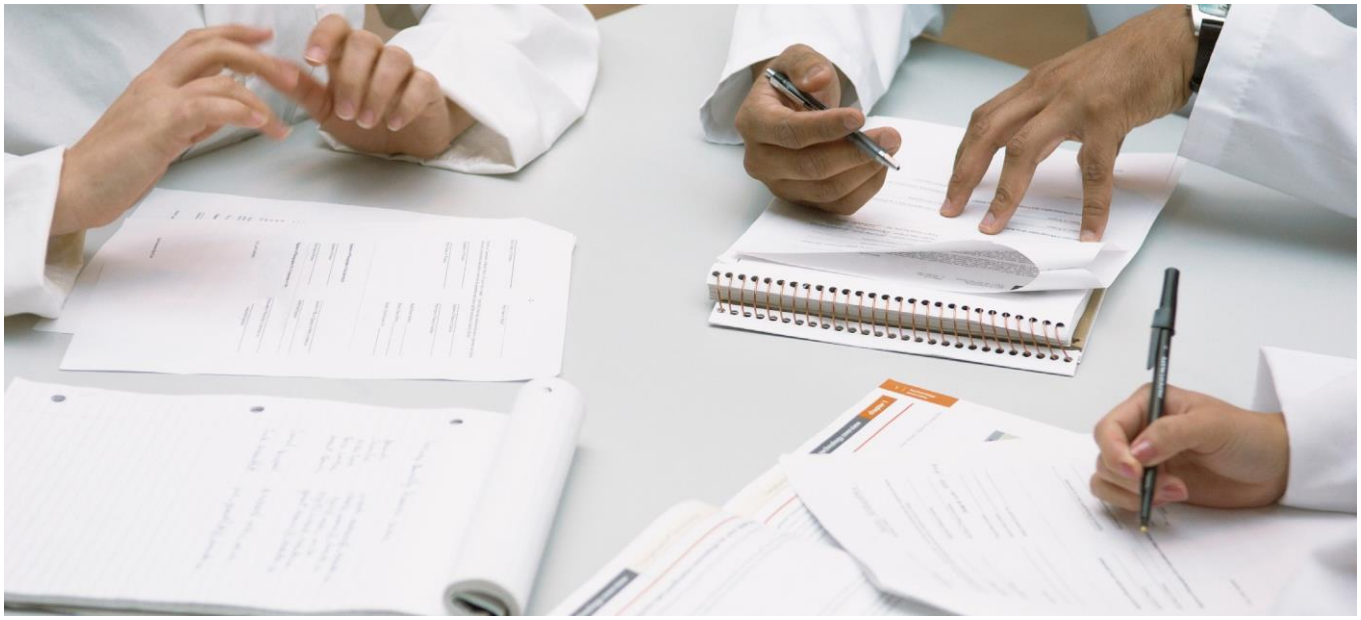
Despite the small numbers of patients required for registration, there are several factors that need to be addressed. Many of these should be discussed from the outset with medical experts and regulatory authorities, for example, appropriate clinical endpoints and biomarkers and the appropriateness of using placebos in subjects facing severely reduced life expectancies. Other areas that need to be considered include harmonisation of international registries to make sure the same data is collected and the fostering of better public-private partnerships to assure necessary investments and resources in the development of treatment strategies.

There are many possible future challenges: regulatory authorities may decide to raise the standards or stop helping companies in the face of increasing number of pharmaceutical companies approaching orphan indications and payers may start to baulk at the increasing money being spent on orphan diseases where previously patients with these conditions could only be treated symptomatically and because of their limited lifespan, for a relatively short time.

More and more conditions previously considered untreatable now can be treated and lead to longer survival for these people. As the number of pharmaceutical options for orphan indications increases, the regulatory standards may become more stringent, raising the costs of the development programme.

There may come a time when society will consider these drugs to be cost-effective but simply unaffordable. In each case, a strong economic argument will need to be built that ensures that despite the high price of the drugs they represent and are seen to be good value and they can generate a net saving for the health care systems.

There is no doubt that orphan drug development is highly suited and can be extremely profitable to smaller companies and larger ones looking to expand their product portfolios. While the costs and time needed to get to registration may be substantially smaller, there are many risks that can be mitigated by a clear understanding of the process and changing environment.



Introduction

There is a growing need to achieve a better awareness and clarity concerning the development of Orphan Medicinal Products. The first Worldwide Orphan Medicinal Designation Workshop (1) held in London in March 2014, brought together regulatory representatives from the three large regions, EU, US, and Japan, which have legislation to foster development of medicines for Rare Diseases. The purpose of the meeting was to present their respective designation systems, the post-designation incentives programs accessible after obtaining designation, and the grants programs designed to foster research and development of Orphan Medicinal Products.

There are an estimated 7,000 rare diseases (2) affecting approximately 350 million people globally. Eighty percent of rare diseases are of genetic origin and approximately 50% of the patients affected are children. The conditions targeted are mostly chronic and degenerative or life-threatening rare diseases in serious need of treatment, e.g., Duchenne muscular dystrophy (3), which affects about one in 3,600 new born boys causing muscle degeneration and eventual death by 30.

A better understanding of the underlying science has enabled viable approaches to be identified. In some cases this is an understanding of the genetic basis for the disease which has led to subtypes of disease to be identified, for example over 500 different Cystic Fibrosis (CF) mutations exist but Delta F508 mutation CF occurs in 70% of all defective genes (4). With this attitude change, orphan drug development can be a commercially viable option. This has led to a change on the R&D landscape.

Alexion's Soliris (5) was approved in 2007 to treat paroxysmal nocturnal hemoglobinuria and costs US$ 440K per patient per year yet private insurers and national health insurers are willing to pay because the treatment transforms patients' lives. Alexion is expected to make around US$ 2 billion in annual revenue from Soliris in 2014, up from US$ 1.55 billion in 2013, with only a few thousand patients worldwide. So, clearly some orphan drugs can be profitable when treating extremely rare diseases.

The Global Orphan Drugs market is forecast to grow at a compound annual rate of 5.67% over the period 2013-2018 (6). It is projected to reach US$ 105 billion by 2018, driven by a growing population and the number of previously-untreatable conditions that can now be effectively treated (7). However, a 2007 paper (8) suggested that the standard methods of health technology assessment incorporating economic evaluation, showed that orphan drugs do not usually prove to be cost-effective and warned that the current system may not be sustainable in the long term. In 2013 the U.S. Food and Drug Administration (FDA) granted orphan drug designation to 258 drugs and approved 33, compared to 188 and 26 respectively in 2012. Since the enactment of the Orphan Drug Act in 1983 the FDA has given 471 marketing authorisations.

Also in 2013, the European Medicines Agency (EMA) designated 140 orphan drugs out of 200 applications, at almost the same level as in 2012. EMA has granted marketing authorisation to 85 orphan medicinal products, 40% of which are to treat cancer, since the introduction of the legislation in 2000. However, another 66 orphan MAAs were withdrawn and another 10 refused.

Since the legislation of 1993 Japan has given approval of manufacturing and sales to 95 orphan drugs out of 327.

Because of different international laws and regulations the regulatory agencies are making limited progress towards the harmonisation of the procedures for obtaining orphan drug designation and approval. For instance in the EU a rare disease is defined as occurring in around 250,000 people while in the US it affects less than 200,000 and in Japan less than 50,000. Although there are slightly different definitions and mechanisms, the regulatory agencies encourage the discovery and development of orphan drugs with exclusive marketing rights, government financial incentives, technical advice, and shorter development timelines.

Pharmaceutical companies are now targeting precise subtypes of a disease and ask that each be counted as an orphan disease (9). In 2013 FDA has granted orphan drug designation to at least 21 lymphoma treatments on the basis of the immune cell affected. To try and discourage this potential 'salami slicing' of a disease, the FDA is urging applicants to provide scientifically plausible evidence for each subtype.

The EU also encourages the applications for orphan medicinal designation for rare diseases other than just in rare cancers, such as extremely rare genetic diseases numbering less than 1000 patients.

Investors in the development of treatments for orphan conditions should be aware of the inherent obstacles to capture their potential value. Important challenges to overcome include the choice for biological targets, difficult clinical study execution, complex regulatory process, and also the changing reimbursement environment.

In this article, we will address why both small and big pharmaceutical and biotech companies want to develop drugs for rare diseases, whether orphan drug development is commercially viable, what needs to be optimised in orphan drug trials, and the overall challenges of orphan drug development. These factors need to be carefully considered before engaging in orphan drug development so as to avoid the pitfalls leading to so many orphan drug designations not progressing to marketing authorisation. It is equally crucial that the drug developers understand how this market is changing and steps they can take to mitigate the risks.

Orphan indications are inherently attractive to small drug companies

Small companies generally have been in disease areas that large companies have had little or no interest in because of the size of the patient population and the difficulties of making a return on investment of sufficient interest. In the past, large companies were more driven by finding the next 'blockbuster' to fuel their large and expensive R&D engines and by the expectations of their shareholders.

Small companies can exploit the science emerging from academic research institutes without the need to build an extensive research and development infrastructure – and indeed they may have been formed specifically for that purpose as Universities have become increasingly astute in supporting and fostering the exploitation of their research product.

There is little or no need to build a development organisation with a global reach. Increasingly, Clinical Research Organisations (CROs) offer the full range of services required to move a prospective drug through all phases of development. However, the challenge will continue to be to design and execute cost effective development programmes.

The potential for a rapid progression to market may make an orphan drug development an attractive option for a venture capital company as it offers the possibility of an early exit with significant return on their investment.

There is significant commitment and assistance available from the regulatory authorities to support small organisations which may lack the experience and expertise to design a complete development programme. It is the only area where the regulators are prepared to commit to agreements on the requirements for registration. By entering into discussions at an early stage with the regulators and, of course, if the data is supportive, the probability of success is higher and the likelihood of significant regulatory delays reduced.

The size of the clinical trials and, therefore, the development time and costs are a small fraction of that required for a 'blockbuster'. For drugs targeted at a primary care disease, individual Phase 3 trials may require thousands of patients to be enrolled at a cost of several hundred million dollars. In contrast, an orphan drug can secure regulatory approval with data from around 100 patients simply because running larger trials may not be feasible. As a corollary, it becomes a realistic proposition for a small company to secure funding to cover the entire development programme. In addition, in many cases there are active and very supportive disease advocacy groups, for example the Cystic Fibrosis Foundation, that may be able and prepared to support the research and development programme.

The population of disease experts around the globe for orphan conditions is small – it is relatively easy to engage them as a small company, especially if few if any other companies are interested in the area. Performing clinical trials with such a captive and motivated group becomes a very manageable proposition for a small company.

The market exclusivity available for a successful orphan drug removes the threat that a big player with a significantly larger commercial organisation can out-compete the smaller company. In essence, a new effective therapy can reach and retain almost 100% of the available patient population at least for the duration of the exclusivity period.

The limited number of patients usually results in all patients being under the care of a very limited physician population, resulting in low marketing costs as there is no need for major advertising campaigns or a large sales force. Every physician likely to prescribe the drug can be easily identified and targeted with all the information they require.

What about the future?

Large companies are becoming interested in orphan approaches especially if, as exemplified by Gleevec, this can be built on after the initial filing. Indeed, it may be that larger companies may only progress drugs with the potential to treat multiple indications or they can build a franchise in a disease area by developing several drugs for specific sub-populations (e.g., CF). The key will be to understand out how to do this cost-effectively given their significant investment in infrastructure.

Small and medium sized companies will continue to see orphan drugs as significant opportunities.

Price will continue to be a key issue. The high prices that can be achieved which make orphan drugs so attractive will need to be justified by significant benefits to both the patient (in terms of treatment options and efficacy) and the health care providers (in terms of a reduced burden on the health care system). In the UK, the National Institute for Health and Clinical Excellence (NICE) plays an important role in balancing the benefits provided to individuals or small numbers of patients with the costs and impact on the overall provision of health care. High priced drugs for a small number of patients that do not have sufficient benefits in terms of cost per quality of life-years (QALYs), such as some of the recent oncology therapies, will not be approved.

Why are Big Pharmaceutical Companies Developing Orphan Drugs?

The number of drugs in the pipeline from big pharmaceutical companies has diminished since FDA market approvals peaked at 145 during the 1994 - 1988 period, and declined to 69 between 2004 and 2008 (10). In addition, many 'blockbuster' drugs are coming off patent resulting in a massive rise in generics and consequent loss in revenues by the originating company. Alongside this, many countries have developed legislation to encourage generally smaller companies to develop drugs for rare diseases. This made sense as large companies previously were simply not interested in drugs for small populations seeking only the next 'big one'. Seeing their revenues disappearing into the pockets of generic companies as well as the high prices companies developing for rare conditions were getting for their products have resulted in a massive move for larger companies to get involved.

What are the perceived benefits of developing drugs for rare diseases for big pharmaceutical companies?

In the US and other countries there are tax credits, a waiver of regulatory fees, funding grants for clinical trials and a period of exclusivity for the marketable medicinal product. Clinical trials are smaller (and cheaper) and some can be fast tracked, with an increasing proportion significantly benefiting children with rare diseases. There are additional benefits because of the small number of patients: they are often treated in a small number of centres so it is easy to identify and market to key opinion leaders, and patients are highly motivated to participate in studies.

On the business side, incentives have included premium prices, reduced marketing costs, increased reimbursement possibilities for chronic unmet medical need and a longer exclusivity. A corollary of providing treatments for rare, usually rapidly fatal conditions, is of course that these patients survive much longer and continue to need more drug.

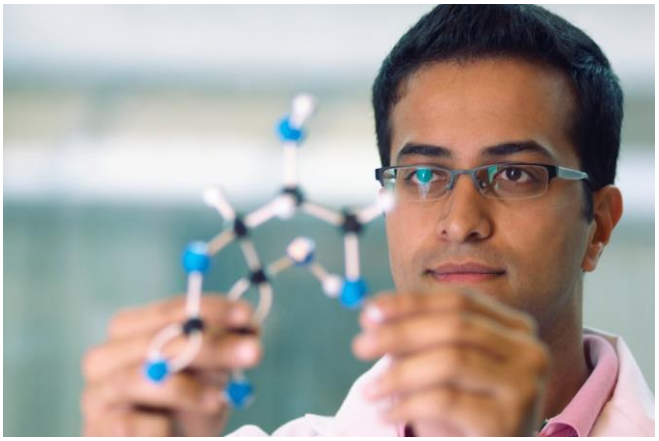
Gross profit margins are massively higher than for the industry as a whole (80% vs 16%) (10). Of the 43 branded drugs having global annual sales of greater than US$ 1 billion 18 were approved solely as orphan drugs in the US (11), and of these 11 reached 'blockbuster' status (defined as one that generates annual sales of at least US$ 1 billion) within the seven-year orphan drug market exclusivity period. Further, as a high proportion are biologics, the manufacturing process might be quite complex thus creating another barrier to entry. So, for all of these reasons, it is unsurprising that big pharmaceutical companies have become intensely interested in this sector.

There are caveats in this business model

Small biotechs developing drugs for orphan conditions are not seen in the same way as large pharmaceutical companies. Because of the small numbers of patients, they often develop close relationships with the patient groups and the patients themselves. Large pharmaceutical companies, on the other hand, may find it more challenging to develop such close relationships given their intimidating size and reputation for being very commercially driven.

Once it is clear that profits can be made, it does not take long for alternatives to appear after the exclusivity period is over particularly for old drugs that have been'repurposed' for an orphan condition. It is essential that reimbursement and future market and environmental conditions are considered before the start of drug development. Failure to do this inevitably leads to increased risk of failure.

There is increased resistance to the high prices for new drugs. The recent example of Sovaldi (sofosbuvir) for hepatitis C is a case in point where the cost of the 12-week therapy is around US$ 84K which can rise to US$ 150K due to other drugs taken as part of the regime. There appears to be no doubt as to its efficacy but payers are saying that while it might be cost-effective, it is simply not affordable. While hepatitis C might not be considered an orphan condition, it is clear that it might lead payers to reject high price drugs as a whole.



Is Orphan Drug Development Economically Viable?

Recent history shows that orphan drugs have the potential for significant revenues if they are new, effective treatments in areas of unmet medical need. And given the benefits already outlined – reduced development time and costs, lower regulatory hurdles, good access to patients and lower sales costs, potentially higher chances of regulatory success – is there now a compelling financial case to develop orphan drugs?

The following valuation example is provided as a hypothetical illustration only to outline potential commercial value. We use a discounted cash flow methodology with forecast costs and revenues. For simplicity it reflects a single large molecule candidate entering Phase 1 in development, and we take into account the probability of success at each step. As a comparison, we perform a similar valuation on a traditional 'blockbuster' approach of a primary care drug in developed markets. Some of the key assumptions are listed below:

	Development Cost	Probability of Launch	Launch Date	Ramp up to Peak Sales	Peak Sales P/A	LOE	Ramp Down of Sales Post LOE	Cost of Manufacture	Cost of Sales
Ophan Drug Base Case	$41M	5%	2021	2yrs	$300M	2031	5yrs	20% of sales	$5M
Traditional Blockbuster	$390M	5%	2022	5yrs	$1.5B	2032	1yrs	20% of sales	$300M

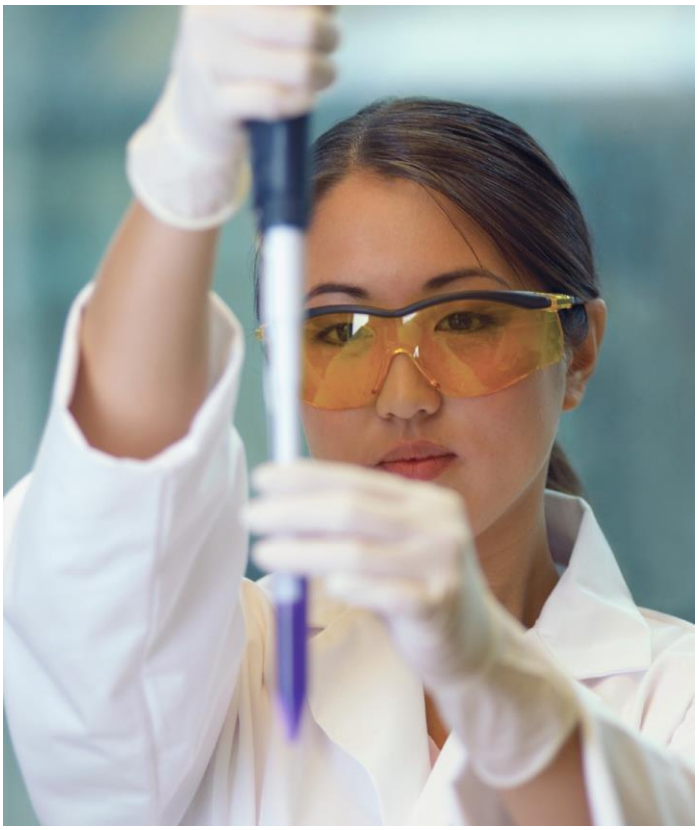
Legend: LOE = Loss of Exclusivity. The period of exclusivity has been assumed to be 10 years for the purpose of this example.

Discount rate: 15% (16)

Results are presented below with the traditional 'blockbuster' approach and the orphan drug"base case" example listed first. The valuation metrics shown here are firstly, Net Present Value (NPV), derived from a whole life profit and loss account and reflecting future development, sales and manufacturing costs, and future revenues derived from sales and secondly, a NPV risk adjusted for the probability of success at each phase, or RNPV.

The RNPV value indicates a NPV we can expect to realise on average across the downstream outcomes – for example, failure at Phase 1, failure at Phase 2a, and so forth all the way to a successful launch. The RNPV will clearly increase as the drug moves forward and development risks are resolved. Looking at the orphan drug "Base Case" there is a low, but positive RNPV and substantial NPV. This can therefore be regarded as a valuable commercial proposition, but perhaps not one with large commercial potential.

With the 'blockbuster' example there is a similar RNPV figure indicating a likely positive return, but a much more attractive NPV figure – indicating that there is an increased risk/reward investment. The return is very attractive but the increased development and sales costs imply higher risk. Exploring some of the key sensitivities generates some "what if" scenarios.



Scenario	In Phase	Dev Costs $M	Peak Revenues $M	Discount Rate	Launch Year	NPV $M	RNPV $M	Notes
Traditional Blockbuster	1	390	1500	15%	2022	499	14	
Base Case	1	41	300	15%	2021	254	11	
Low Revenue	1	41	150	15%	2021	119	3	
Double Revenue	1	41	600	15%	2021	525	25	
Low Discount Rate	1	41	300	10%	2021	463	22	
1 Year Acceleration	1	41	300	15%	2020	303	13	
In Phase 2a	2a	38	300	15%	2020	301	27	Probability of launch 10%
Successful Phase 2a	2b	33	300	15%	2019	356	185	Probability of launch 51%

To start generating an attractive valuation (i.e., double digit RNPV) revenues of US$ 300 million or higher are needed for an orphan approach in Phase 1. The “Base Case” example is at the lower end of what might be seen as commercially attractive, and reducing the revenue to US$ 150 million per year gives a “break even” example. Revenues of US$ 600 million increase the attractiveness of the opportunity, as does reducing the discount rate to 10% (this can be seen as a rate more aligned to a large, stable organisation). A 1 Year acceleration gives only modest increase in value from base case, as does assuming the candidate is in Phase 2a. But a successful Proof of Concept (POC) resolves significant risk for relatively little development cost and gives a substantial increase in value - a value inflexion point for this particular example. In fact the RNPV moves from US$ 11 million to US$ 185 million – a 16 fold increase. Given this, Phases 1 through to POC might represent an attractive investment for funding groups with shorter time horizons than say, investing in the entire development path to launch.

It can be seen therefore that the benefits of faster and cheaper development, reduced sales cost footprint, increased probability of regulatory success can go a good way to offsetting potentially lower sales. And there is some evidence to suggest that orphan drug sales are catching up with non-orphans (12).

Of course each opportunity is different and would need to be examined on a case by case basis, but by carefully selecting those orphan opportunities that have the potential for significantly changing medical practice and therefore commanding high prices and in turn generating significant revenues, we see that there are attractive investments even for candidates in early stages of development.

Big pharmaceutical companies with large and costly R&D organisations and large fixed cost overheads may require more significant revenue opportunities than orphan drugs can provide in general. They might currently be seen as a welcome addition in the search for the next big revenue drug though they are unlikely to be a replacement for it. Larger pharmaceutical companies are moving toward a more fragmented and outsourced R&D model but may not be ready to rely solely on these niche opportunities.

However for smaller companies, funding groups and biotechs there can be great opportunity particularly given the current appetite for licensing, acquisition and funding for orphan drug development from big pharmaceutical companies for later stage opportunities.

What Needs to be Optimised in Orphan Drug Trials?

The drug developer is expected to engage with the regulators in discussions from inception as medical experts and regulators often fail to grasp poorly understood disease processes.

Potential issues that need to be addressed with regulators from the outset

- Medical plausibility from the clinical data for the expected treatment benefit
- Patient recruitment strategies supported by rare disease physician networks and patient advocacy groups
- Use of placebo in subjects facing severely reduced life expectancies
- Outcome measures, identified biomarkers of natural disease progression, endpoints, study durations linked to the decline in patients
- Appropriate choice of adaptive study designs, if allowed by the sample size and statistics, to shorten development time and cost
- Risk Evaluation and Mitigation Strategies for the orphan medicinal product
- Stable formulation and sufficient supply for the duration of orphan drug testing

What needs to improve in orphan drug development

- Standardisation of pan-regional registries of rare diseases to ensure well-defined homogeneous populations
- Aggregation of universally-standard data about bio-specimens from many institutions to build effective sample sizes (13)
- Design of studies enabling to determine the effectiveness of the selected treatment in subjects living with different stages of the rare disease (14)
- Fostering of better public-private partnerships to assure necessary investments and resources in the development of treatment strategies. An example of this would be the Innovative Medicines Initiative, a joint undertaking between the EU and EFPIA, is Europe's largest public-private partnership aiming to improve the drug development process by supporting a more efficient discovery and development of better and safer medicines for patients.
- Creation of multi-disciplinary bio-informatics hubs to map the rare diseases. The Sheffield Bioinformatics Hub is a new venture jointly sponsored by the Sheffield biology departments (MBB, APS and BMS), two Medical School research centres (SITraN and Sheffield Cancer Research Centre) and the NHS (Sheffield Children's Hospital Trust).

Possible Future Challenges in Orphan Drug Development

Drug developers will end up with higher costs and studies will take longer and become more complicated if the regulators, because of the increasing number of pharmaceutical companies approaching orphan indications, decide to raise the regulatory standards.

Patient advocacies already sit on regulatory committees. They are becoming more educated and able to make inroads into research to find a treatment and, in the absence of this, finding ways of improving well-being and general quality of life. They employ Scientific Consultants who bring pharmaceutical-type thinking in helping them write grants, peer-review scientific proposals, evaluating interesting molecules from academia, and preparing development plans. For example, advocates for Duchenne Muscular Dystrophy (15) have recently started a petition for the White House to encourage the FDA to provide an expedited review for the approval of safe and effective therapies.
Patient confidentiality will become increasingly hard to maintain as orphan drug companies often interact directly with patients on a personal level.

Payers may start to limit access to orphan drugs. This is a particular danger for those drugs that offer limited differentiation from other drugs for the same condition, in response to budgetary pressure and they may request convincing data to justify that the orphan drug is worth the price. Therefore, in countries where patients co-pay, the patients will end up paying higher prices.

References:

1. Worldwide Orphan Medicinal Designation Workshop European Medicines Agency, London, United Kingdom, 10 March 2014
2. Rare Facts and Statistics. www.The Global Genes Project. Accessed 02 May 2014
3. Learning about Duchenne Muscular Dystrophy. www.genome.gov › ... › Specific Genetic Disorders. Accessed 02 May 2014
4. Types of Cystic Fibrosis. www.medicinenet.com › ... › lungs a-z list › cystic fibrosis index. Accessed 02 May 2014
5. Alexion climbs on strong Soliris sales forecast. ww.businessweek.com/...30/...soliris-sales-fore... Accessed 02 May 2014
6. Global Orphan Drugs Market 2014-2018. www.researchandmarkets.com › ... › Pharmas. Accessed 02 May 2014
7. Global orphan drugs market: US$ 105 billion industry forecast by 2018. www.reports.pr-inside.com/global-orphan-drugs-market... Accessed 02 May 2014
8. Drummond MF, Wilson DA, Kanavos P, Ubel P, Rovira J. Assessing the economic challenges posed by orphan drugs. J Int J Technol Assess Health Care. 2007; 23(1)):36-42
9. Reardon S. Regulators adopt more orphan drugs. Nature. 2014 Apr 3;508(7494):16-7
10. Phillips MI. Big Pharma's new model in orphan drugs and rare diseases. Expert Opinion on Orphan Drugs 2013;1(1):1-3.
11. Cote T, Kelkar A, Xu K, et al. Orphan products: an emerging trend in drug approvals. Nat Rev Drug Discov 2010;9(1):84
12. Meekings KN, Williams CSM, and Arrowsmith JE. Orphan drug development: an economically viable strategy for biopharma R&D. Drug Discovery Today 2012; 17 (13/14):660-664
13. Biobanking Advancing Biorepositories with Data Science 2014 5AM Solutions
14. H P Selker, K A Oye, H-G Eichler, N L Stockbridge, C R Mehta, K I Kaitin, N E McElwee, P K Honig, J K Erban and R B D'Agostino. A Proposal for Integrated Efficacy-to-Effectiveness (E2E) Clinical Trials Clinical Pharmacology & Therapeutics (2014); 95 2, 147–153.
15. 100,000 People Sign Duchenne Petition. Rare Disease Report March 25, 2014
16. News in Avance – valuation in Life Sciences. January 2008 no. 1

Volt Pharma Associates (**VPA**) is an associate network of pharmaceutical professionals with many years of practical experience in their chosen fields. VPA supports life sciences companies, investors and academic institutes in developing new products, technologies and innovative approaches. VPA's aim is to solve their clients' product research, development and commercialisation challenges so they can achieve their business goals.

VPA is part of Volt, a global group with over 200 offices covering Europe, the Americas and Asia, generating annual revenues in excess of US$ 2 billion. Volt delivers best in class Talent, Technology and Consulting services to its customers around the globe.

For further information, please contact:

Claude Houet
Dipl. Rer. Pol (MSc)

Head of Practice
Pharmaceutical and Life Sciences Industry

claude.houet@volt.eu.com
www.vpa.eu.com
+49 1726340202

About the Authors:

Graham Finch, BSc, BEng, MSc

Graham leads the VPA POEM (opportunity evaluation) practice and is providing strategic and analytical guidance to Research and Development and Business Development investment decisions. He has a background in asset and portfolio strategy, commercial analysis and valuation, business development search, evaluation, due diligence and deal terms for licensing and acquisition, market and customer analysis, investment and risk analysis.

John Bennett, BSc, PhD

John is a VPA Lead Associate and leads the VPA P3M practice. He has 25 years of experience in drug discovery and development and provides consulting services in portfolio, programme, project and finance management areas including developing Strategy and operational planning frameworks, working with individual drug project teams to craft development strategies and operational plans and improving annual portfolio review and planning processes. He has also extensive experience of working closely with project teams to improve their effectiveness.

Mauro Placchi, Dott. Chimica (MSc)

Mauro leads the VPA clinical & regulatory services practice he is a clinical development consultant with 24+ years of experience, skilled in all aspects of Phase I-IV clinical trials including planning, organizing, implementing, leading, controlling, and reporting. Successfully worked across multiple technology platforms (small molecules, therapeutic proteins, mAbs, devices) and in differently targeted environments (prescription drugs, consumer products).

Dr. Richard Phillips, MBBS, DipPharmMed, MBA

Richard leads the VPA commercialisation (VPAC) practice. He brings a wide background in clinical studies, economic analyses and meta-analyses following nearly 29 years in the pharmaceutical industry as well as wide experience in presenting clinical and health economic studies both in print, at symposia and for training purposes. He has worked with several companies in the health technology assessment, pricing and reimbursement and market access fields. He is the author of numerous market, data & literature reviews and core-value documents.

"Committed to delivery through collaboration"