ProFactor Pharma Ltd

Transforming treatment of haemophilia

Executive Summary

May 2018

INVESTMENT OPPORTUNITY:

> Investment Required: £2.1m

>Valuation : £4m pre money

> Cornerstone Investor Secured: £600k +

>EIS qualifying

PURPOSE OF INVESTMENT:

Funds will be used to:

1. Process yield optimisation and scale up in advance of pre-clinical toxicology studies leading to Clinical Trials Approval (CTA) in 2020

2. Generate Fully GMP Compliant Master and Working Cell Banks (M/WCB)

3. Complete toxicology studies

The Problem..

- Factor VIII is normally produced in the liver but a dysfunctional gene in males, prevents this in haemophiliacs who have limited or no capacity to produce this. This results in spontaneous, uncontrolled bleeding, particularly into joints and internal organs resulting in severe pain and disfigurement. Without treatment haemophiliacs typically die in their teens.
- Of 600,000 potential haemophilia A sufferers worldwide, only 150,000 are treated with Factor VIII* and the number requiring treatment to stay alive is growing constantly.
- Injections are required two to three times per week to prevent bleeding or following injuries causing bleeding.
- Supply is production constrained and usage is cost constrained. The global market, until very recently was dominated by major players such as Baxter, Pfizer and Bayer.

*World Federation for Hemophilia Reports on the Annual Survey 2016.

UNTIL RECENTLY GLOBAL SUPPLIERS OF FACTOR VIII WERE:

> Baxter (Baxalta now Shire)
>Bayer
>Pfizer (Wyeth)
>CSL- Behring

Their production is highly capital intensive and expensive. Patents have now fallen, so rather than risk vast capital expenditure, they have focused on a fixed quantity of product for the highest price markets, which generate $billions for them.

THE OPPORTUNITY:

The key patents for rhFVIII have now fallen, opening the market to new suppliers.
Novo Nordisk, Octapharma and Biogen-Idec are now bringing Factor VIII versions to the market. This validates the opportunity for a successful, low cost manufacturer of rhFVIII to enter the market. Since Biogen-Idec entered they have secured sales of $300m in 2015 and $543m in 2016.
PFP have a process that can be scaled and repeated globally.

The Solution..

- There are two types of Factor VIII: extracted and purified from human blood (plasma derived) or manufactured using recombinant technology- rhFVIII (Factor VIII is engineered into cells that act as a biological factory)
- ProFactor Pharma **have developed a low cost recombinant factor VIII** for the treatment of Haemophilia A, using a proprietary high expressing cell line. PFP's competitive edge is a bioprocess delivering high yields using low cost disposable technology resulting in a lower COGS.
- PFP's rhFVIII has been proven as indistinguishable from existing products in the marketplace, de-risking the clinical trial process.

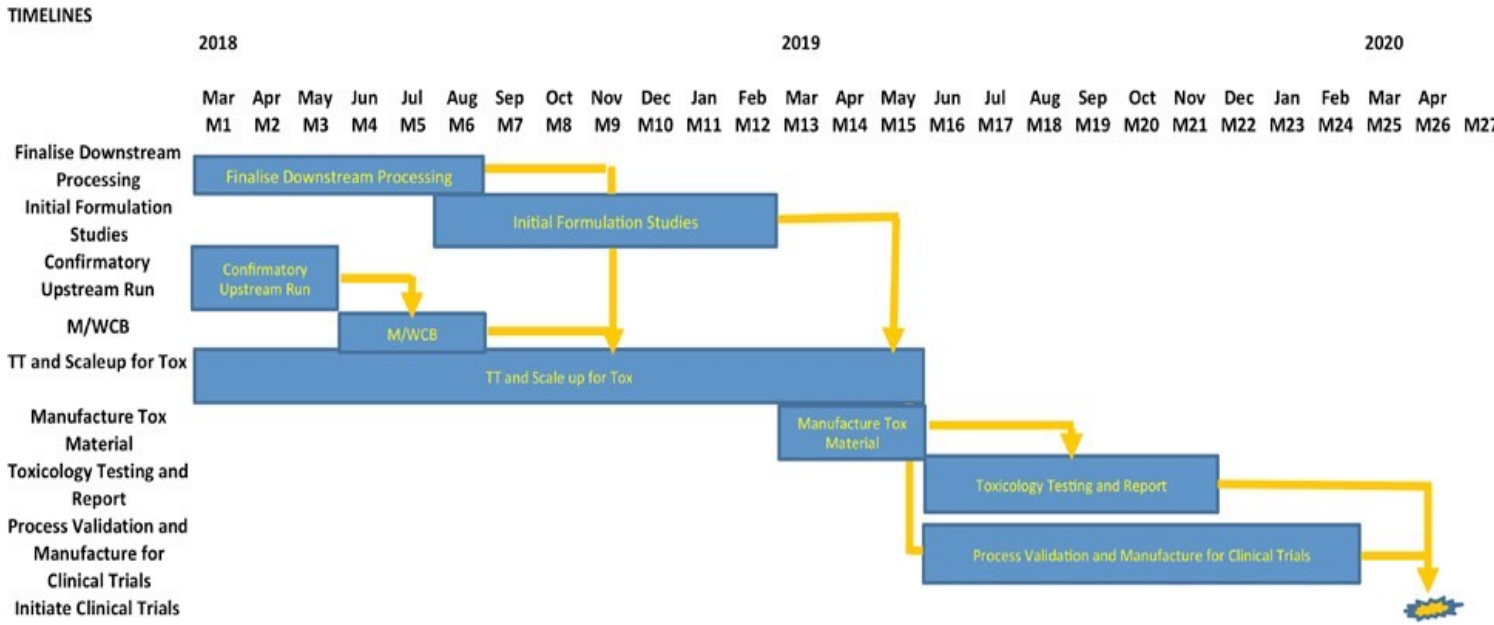
The Market..

- **Annual sales of Factor VIII now exceed $8bn**, of which recombinant sales were **$5.4bn*.** This will continue to grow as we know there is a huge undersupply and unmet need.
- Price and undersupply is currently a major issue in BRIC and Eastern Europe. Despite the affluent in China and India being able to afford treatment, they simply cannot source sufficient product.

* Sales of Recombinant Therapeutic Antibodies and Proteins – La Merie Publishing; March 2017

KEY FACTS:

- PFP's rhVIII has been verified through characterisation work undertaken by the University of Surrey and the University of Dundee, as indistinguishable from ReFacto, the Pfizer product.
- Clinical trials are substantially de-risked in biosimilar products, as outcomes are expected to be similar to those of existing products, that have completed the process.
- Ingenza are cornerstone investors and are also supporting the company with resource and facilities.



- As this is a known active, initial clinical trials only require tests on 50 patients for 50 treatment days. In order to minimise the funding requirement leading to product sales and profits, initial trials will focus on adult PTPs giving the quickest route to Marketing Approval. Further trials, post launch, for which there is an obligation to the EMA, will expand approval and usage to under 12 years and previously untreated patients.
- Allowing time for patient recruitment and approval of the results we anticipate market launch in 2023.

SUMMARY:

- Vast Global Market ($8bn pa)
- Product that is stable, scalable and competitive.
- Low production cost allows for aggressive pricing structure.
- Market is under supplied

- INVESTMENT OPPORTUNITY:

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The Opportunity..

- PFP's process offers significant costs reductions and is highly scalable which allows them to go to market with an aggressive pricing structure (£0.20 per IU vs average market price of £0.60 per IU).
- Through the use of CMO, sales are projected at **~£100m** in 2023, and increasing to **~£600m in 2024,** with increased production capacity.
- PFP have patented their core technology of upstream processing and have filed worldwide. A further patent filing has been made on the downstream process.

Exit Opportunities..

- It is expected that the company will become an acquisition target, potentially even before they go to market.
- An IPO is also a possibility within the next two- three years.