

22<sup>nd</sup> November 2018

## Shareholder Update – Frequently Asked Questions

Dear Shareholders

Since last week's announcement we have received a number of questions about the results of the Company's Phase 2 VF00102 study. We believe the answers to these questions will be of interest to all our shareholders and have prepared this "Frequently Asked Questions" document for information.

Early closure of all study activities is progressing well as we work closely with our collaborators to ensure all regulatory requirements are met. Finalising these activities will enable the Company to focus on maintaining its financial resources and the existing intellectual property portfolio while the Board of Directors considers the options for this intellectual property.

Thank you for your continued support throughout this process.

Sincerely,



Dr Rosalind Wilson

Chief Executive Officer

---

## Frequently Asked Questions

### 1. How do the results of VF00102 compare with the previous VitroCARD study?

Both studies showed very similar improvements in healing. In the VitroCARD study the reduction in wound area was 54.0% and the rate of full healing was 35.6%. In VF00102 – in which all groups received the same standard care – the wound area reduction was 55.7% for placebo, 61.0% for VF001 low dose and 57.4% for VF001 high dose; and the rate of full healing was 35.8% for placebo, 40.0% VF001 low dose and 35.8% VF001 high dose. It should be noted that the VitroCARD trial did not have a placebo or control arm and all participating physicians and patients knew that VF001 was being used.

### 2. In the VitroCARD study the product appeared to be effective for VLU healing. Why was this result not replicated in VF00102?

The VitroCARD study provided a very encouraging signal that adding VF001 (previously known as VitroGRO-ECM) to standard care would improve healing of venous leg ulcers (VLU); and the Phase 2 VF00102 study was designed to more thoroughly evaluate this signal.



VF00102 was an appropriate test of VF001 for the Phase 2 stage of development, where results in patients receiving VF001 added to the best currently available care (standard care i.e. dressings and compression bandaging) were compared with patients receiving standard care and a matched placebo. The goal of VF00102 was to assess the added benefit of VF001 and the results showed that, while patients in the trial experienced healing of their VLU, there was no clinically meaningful improvement in healing when VF001 was added to standard care.

These results indicate that well-managed standard care is an effective treatment for many VLUs. This finding is consistent with the balance of wound care clinical research evidence where, to date, additional treatments have not been consistently shown to be more effective than compression bandaging.

It is well recognised that, in developing new treatments, the Phase 2 stage is associated with the highest risk – a majority of products are not able to confirm the potential promise shown in earlier stage preclinical or clinical studies, with a success rate of only 30% for moving from Phase 2 into Phase 3.

### **3. Could this result have been detected sooner?**

When a new treatment is developed, early stage experiments and early phase trials can provide an indication of effect; however, as development progresses more rigorous clinical studies are needed to fully understand the benefits and risks. This is a standard and well-accepted principle of drug development.

Earlier tests of VF001 provided a very encouraging signal of activity and VF00102 was the appropriate next test of VF001, at the Phase 2 stage of development. The VF00102 trial also followed clear guidance for industry from the FDA for trials at this stage of development. The study was fully blinded so that the results would only be known once treatment was fully completed.

### **4. Is it possible there has been any error in the process of unblinding and analysing the data?**

VF00102 was conducted to a very high standard, as the results had the potential to form the basis of medical device approval in the EU and a further investment decision for Phase 3 in the US. The trial and analyses were managed by PAREXEL, a leading clinical research company, with extensive experience in conducting these “gold standard” clinical trials. The process of unblinding and data analysis involves multiple quality checks before results are released and no issues were identified during this process.

### **5. Is it possible there was a problem with the product or its manufacturing?**

VF001 for the study was manufactured to established industry standards – Good Manufacturing Practice, GMP – and there is no evidence to suggest any problems with either manufacturing or product quality.

GMP standards require appropriate independent quality checks and balances; in addition, critical suppliers were audited by Factor Therapeutics against industry standards and found to be fit for purpose; and the investigational product was shipped and stored under correct conditions.



Consistent product quality was demonstrated throughout manufacturing and the trial, including batch release procedures to ensure batch manufacture and product specifications were met (this includes assays – tests for potency). Batches used in the clinical trial were all tested in a stability study, which is an FDA requirement for studies run under a US IND. The product met the specification for the duration of this stability study until the trial was halted. We have no reason to doubt the integrity of the product used in the trial.

#### **6. Will there be further data analysed from VF00102?**

No further analyses will be conducted.

While the results of the study did not show a benefit for VF001, the design of VF00102 and the data it has yielded are extremely valuable for the field of wound care clinical research and the study results will be written up for publication.

#### **7. What happens next in relation to the study?**

Following the results, the decision has been taken to close the study early and patients who are in the post-treatment follow-up phase will be informed by the sites responsible for their care. The study results will be written up for publication.

There is a small amount of product at the sites – and in storage – that needs to be destroyed as part of the regulatory requirements for study closure.

The issue of “waste” through destruction of material has been raised in the past and, while we agree that it is a shame to destroy a product that is expensive and difficult to manufacture, we have a legal and risk-management obligation to ensure that no material is out of our control. Storage and ongoing testing of any remaining product would be very expensive and this is a cost we are not willing to incur with the remaining inventory.

#### **8. Will VF001 be developed for any other uses?**

The decision has been taken to cease development of VF001 in all indications. The results from VF00102 show that VF001 would not be a clinically useful treatment for venous leg ulcer healing and would not be approvable by regulators, as either a medical device in the EU or a biologic in the US. The development risk for VF001 in other indications such as diabetic foot ulcer is also substantially increased as a consequence of this very well-controlled trial.

The Company’s immediate focus is to ensure an orderly and efficient early closure of VF00102 and to halt development of VF001 in other indications; to focus on maintaining the Company’s existing intellectual property portfolio; and to conserve the remaining financial resources to enable the Board of Directors to evaluate the remaining options for the company in a timely fashion.

#### **9. Will the Company’s other pipeline products be developed?**

The pipeline comprises an ocular programme (for chronic eye wounds) and early stage discovery of new pipeline molecules (the VF00X programme). The ocular programme was assessing VF001 and two other clinical candidates and this has been halted; and no further work will proceed with VF00X at this time. The Company’s existing intellectual property portfolio will be maintained while the Board considers options for this intellectual property. Historically the Company’s investment in these



other areas was modest and it is not evident that there is a strong fit with very early-stage programmes and the financing reality of a public company.

**10. Why was Taylor Collison appointed as a corporate advisor?**

After a thorough international search, Taylor Collison was engaged to provide transaction advisory services in preparation for the readout in anticipation of a positive result which, unfortunately, did not eventuate.

**11. What is the status with the Company's discussions with potential partners?**

All potential partner companies with whom the Company had an ongoing relationship have been advised of the results from VF00102 and we are responding to questions as they arise. In general, the results of VF00102 were highly anticipated in the wound care field and our prospective partners share our disappointment in the outcome.

**12. What is the Company's current financial position?**

The current focus is on conserving financial resources by ensuring an orderly and efficient early closure of VF00102, halting further development of VF001 in other indications and maintaining the existing intellectual property portfolio.

At the end of the September 2018 quarter the Company had \$4.047 million in cash reserves with a projected spend to 31 December of \$2.489 million. Although we have done everything to stop incurring future cost from the VF00102 trial, there are certain close-out costs remaining and we are working with PAREXEL to finalise these as quickly as possible. The Company's half-year 2018 accounts include a provision of ~\$160,000 for the R&D tax incentive payment and this is based on Australian R&D spend.

The Company will submit an annual tax return in 2019 (after the end of its financial year on 31 December 2018) following a detailed review of the anticipated R&D tax incentive for eligible overseas activities undertaken during the year.

For more information contact Factor Therapeutics at [info@factor-therapeutics.com](mailto:info@factor-therapeutics.com)

---

Erratum: The title of Figure 1 in the announcement of the Phase 2 study results (14<sup>th</sup> November 2018) was incorrect. The correct Figure appears below with the highlighted correction.

Figure 1. Reduction in Wound Area over 12 weeks of Treatment (Mean L, Median R).

