Chief Executive Officer's review of performance

Commenting on the results, Olav Hellebø, Chief Executive Officer, said:

"The past year has been a transformational one for ReNeuron. During the period, we commenced patient dosing in the US placebo-controlled Phase 2b clinical trial of our CTX cell therapy candidate in chronic stroke disability. This was followed shortly afterwards by the announcement of strongly positive preliminary efficacy data from the first three Phase 2a patients in the ongoing US Phase 1/2a clinical trial of our hRPC cell therapy candidate in retinitis pigmentosa. We look forward to delivering further significant clinical data in our stroke and retinitis pigmentosa programmes over the next 18 months.

We are pleased to be working with Fosun Pharma as our partner for China, following the signing of the exclusive licence agreement for both our CTX and hRPC programmes in that territory. We are also encouraged by the level of interest other potential collaborators are showing in all of our programmes, including our exosome technology which is being developed as a novel system for delivering third party drugs.

We look forward to providing further updates on our clinical and commercial progress in the months ahead."

Review of clinical programmes

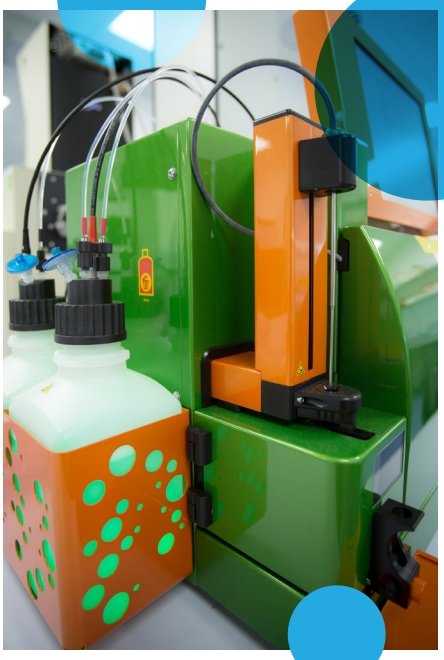
hRPC for retinal disease

During the period under review, and subsequent to it, we have made significant progress advancing the clinical development of our human retinal progenitor cell (hRPC) therapy candidate in the blindness-causing disease, retinitis pigmentosa (RP). A Phase 1/2a open-label clinical trial is ongoing to evaluate the safety, tolerability and preliminary efficacy of our hRPC stem cell therapy candidate in patients with advanced RP. The Phase 2a element of the study, which uses a cryopreserved hRPC formulation, enrols subjects with some remaining retinal function and is being conducted at two clinical sites in the US: Massachusetts Eye and Ear in Boston and Retinal Research Institute in Phoenix, Arizona.

In February 2019, we reported positive preliminary data in the first cohort of three patients in the Phase 2a element of the study, with all three subjects in the cohort demonstrating a rapid improvement in vision compared with their pre-treatment baseline.

In April 2019, further data from the first patient cohort in the study were presented at the sixth annual Retinal Cell and Gene Therapy Innovation Summit in Vancouver, Canada, which preceded the 2019 annual meeting of the Association for Research in Vision and Ophthalmology. In the presentation, it was reported that the first cohort of patients in the Phase 2a element of the study had demonstrated a sustained and further improvement in vision compared with baseline, with a mean improvement from baseline in visual acuity of + 23 letters on the ETDRS eye chart in the treated eye (the untreated control eyes did not show meaningful improvement). An improvement of + 23 letters is equivalent to reading an additional four lines of letters on the ETDRS eye chart, the standardised eye chart used to measure visual acuity in clinical trials.

Olav Hellebø
Chief Executive Officer



An improvement of at least + 15 letters from baseline is considered to be clinically meaningful by the US Food and Drug Administration (FDA), as stated in their recent guidance on gene therapy for retinal disorders. In addition to these objective measurements, all three subjects had also noted a subjective improvement in vision in their treated eye.

Dosing of the second cohort of three subjects in the Phase 2a element of the study is complete and dosing of the remaining two cohorts is in progress. These later cohorts comprise patients with a greater baseline level of visual acuity than those patients earlier in the study, as we seek to assess preliminary efficacy in patient groups with differing levels of remaining vision. The clinical protocol for the study allows for up to 12 patients (four cohorts of three patients each) to be treated in the Phase 2a element of the study.

We expect to treat the remaining patients in the study shortly and to report preliminary data from all treated Phase 2a subjects in October at the American Academy of Ophthalmology 2019 Annual Meeting in San Francisco. These results will form the basis of our future interactions with the European and US regulatory authorities regarding the future clinical development path of hRPC for the treatment of RP. Our clinical programme in RP benefits from Orphan Drug Designation in both Europe and the US, as well as Fast Track designation from the US Food and Drug Administration (FDA).

CTX for stroke disability

During the period, we have continued to progress the clinical development of our CTX cell therapy candidate for stroke disability. In January 2019, we announced that patient dosing had commenced in PISCES III, a randomised, placebo-controlled, Phase 2b clinical trial in 110 patients at up to 40 clinical trial sites in the US.

Patients in the study are treated between 6 and 12 months after their stroke and are randomised to receive either CTX therapy or placebo treatment.

The primary end-point of the PISCES III study is the proportion of patients showing a clinically important improvement (at least one point) on the modified Rankin Scale (mRS) at six months post treatment compared with baseline. The mRS is a global measure of disability or dependence upon others in carrying out activities of daily living and is accepted by regulatory authorities as an appropriate end-point for marketing approval in stroke disability.

Based on current patient recruitment and resource planning, we expect to report top-line data from the PISCES III study in late 2020. We expect the PISCES III clinical trial, if positive, to be one of two pivotal studies required to support marketing authorisations for CTX in stroke disability.

Exosome technology

During the period, we reassessed how best to exploit our CTX cell-based exosome platform to maximise potential near-term commercial opportunities. We are pursuing opportunities to capitalise on the significant scientific and life sciences industry interest in exosomes by forming value-generating business partnerships covering our exosome technology. In this regard, ExoPr0, our first CTX-derived exosome candidate arising from this technology, is being developed as a novel vector for delivering third party biological drugs.

In January 2019, we signed a collaboration agreement with a US-based biopharmaceutical company to explore the use of our exosome technology to create delivery vehicles for synthetic oligonucleotides used in gene therapy. We are in active early discussions with other commercial third parties regarding potential collaboration agreements for our exosome technology.

Chief Executive Officer's review of performance continued

Also in January 2019, new data were presented in conference from a grant-funded collaboration between ReNeuron, University College London and the Cell and Gene Therapy Catapult. The new data demonstrated the feasibility of scaling up the production of our CTX-derived exosomes utilising state-of-the-art bioreactor systems, representing a significant advance towards an industrial scale production process without affecting the quality and consistency of the final product.

Business development activities

Our technologies and therapeutic programmes have increasingly attracted the interest of commercial third parties. During the period, a non-refundable exclusivity fee of US$2.5 million was received from one such third party relating to a potential out-license of our hRPC retinal stem cell technology. As previously announced, this potential licensee ultimately withdrew from the deal for reasons unrelated to ReNeuron's technology.

In April 2019, we announced the signing of an exclusive licence agreement with Shanghai Fosun Pharmaceutical Industrial Development Co., Ltd. ("Fosun Pharma") for the development, manufacture and commercialisation of both our CTX and hRPC cell therapy programmes in the People's Republic of China.

Under the terms of the licence agreement, Fosun Pharma will fully fund the development of our CTX and hRPC cell therapy programmes in China, including clinical development and subsequent commercialisation activities. Fosun Pharma has also been granted rights to manufacture the licensed products in China. In return, ReNeuron received £6.0 million (before withholding tax) on entering into the agreement and will receive up to £6.0 million in near-term operational milestones and up to £8.0 million in future regulatory milestone payments.

In addition, ReNeuron will receive estimated post-launch profit threshold milestone payments of £80.0 million provided all milestones and profit thresholds relating to the licensed products are successfully met, as well as tiered royalties at rates between 12% and 14% on sales of the licensed products in the Chinese market.

We remain in discussions with other commercial third parties regarding potential collaboration and/or out-licensing deals across our programmes.

Other activities

In October 2018, we presented data demonstrating for the first time that our lead CTX cell line can be successfully reprogrammed to an embryonic stem cell-like state and then differentiated along a different path from the original cell line. Importantly, ReNeuron's immortalisation technology remained functional in the reprogrammed cells. These results demonstrate that our CTX cell line could be used to produce new conditionally immortalised allogeneic (i.e. non-donor-specific) cell lines from any of the three germ layers: ectoderm, mesoderm and endoderm. We are now working to develop further new allogeneic cell lines, including NK and T-cells (the cells that can be modified to attack cancer cells), as potential therapeutic agents for out-licensing to third parties.

Summary and outlook

The last year has been a transformational one for ReNeuron. During the period, we commenced patient dosing in the US placebo-controlled Phase 2b clinical trial of our CTX cell therapy candidate in chronic stroke disability. This was followed shortly afterwards by the announcement of strongly positive preliminary efficacy data from the first three Phase 2a patients in the ongoing US Phase 1/2a clinical trial of our hRPC cell therapy candidate in retinitis pigmentosa.

We look forward to delivering further significant clinical data in our stroke and retinitis pigmentosa programmes over the next 18 months.

We are pleased to be working with Fosun Pharma as our partner for China, following the signing of the exclusive licence agreement for both our CTX and hRPC programmes in that territory. We are also encouraged by the level of interest other potential collaborators are showing in all of our programmes, including our exosome technology which is being developed as a novel system for delivering third party drugs.

We look forward to providing further updates on our clinical and commercial progress in the months ahead.

Olav Hellebø
Chief Executive Officer
18 July 2019