TOWARDS A CELL-BASED TREATMENT FOR ANDROGENETIC ALOPECIA IN MEN AND WOMEN: 12-MONTH INTERIM SAFETY RESULTS OF A PHASE I/IIA CLINICAL TRIAL USING AUTOLOGOUS DERMAL SHEATH CUP CELL INJECTIONS

CLINICAL TRIAL PROTOCOL

The primary objective of the study is to assess the local (at treatment sites) safety profile of injections of autologous dermal sheath cup cells (DSCC) at 6 months post-injection compared to control. Secondary objectives were to assess systemic (overall) safety, local safety, and efficacy at 24 months post-injection.

SCREENING

As part of the screening process subjects had their mild to moderate AGA categorized either according to the Ludwig Scale (female) or the Norwood scale (male) and provided blood samples to confirm their health status (i.e. to exclude HIV, HBV and HCV). Once the participants’ health status was confirmed, 2 cm² treatment sites selected on either side of the fronto-parietal scalp region and occipital scalp biopsies were obtained for manufacturing of the cell therapy product.

TREATMENT

Once manufacturing was completed, participants returned to the clinic to receive randomized, blinded injections of autologous replicated DSCC in a carrier medium (verum) in one pre-selected treatment site, and of carrier medium alone (control) in the other treatment site. A proprietary syringe holder was designed to perform injections for the study in a consistent fashion. Each 2 cm² treatment site received a total of 6 injections that delivered 1.0 ml of either verum or control product. Distribution of the 6 injections was controlled by an injection template. All injections were performed by a study investigator. Depth and angle of each injection was controlled by the design of the holder itself. Rather than injecting products as a bolus, they were released as the needle was retracted from the scalp, thereby leaving a line of product at various levels within the dermis. This ensured that cells came in contact with both the hair follicles and the basal layer of the epidermis.

RESULTS

• Screened 30 subjects (16 male, 14 female).
• Harvested 25 biopsies (12 male, 13 female).
• 19 subjects injected (10 male, 9 female).
• 6 (32%) participants experienced a local reaction (mild burning) related to injections of verum while 3 (16%) experienced local reactions associated with injections of control (burning, pyoderma, excoriation).

Local Tolerance (Safety) at 12-months post-injection

• The majority of injections were well-tolerated.

Secondary objectives were to assess systemic (overall) safety, local safety, and efficacy at 24 months post-injection.

CONCLUSION

The safety data collected from study participants 12-months after receipt of injections of verum and control revealed that the RepliCel® treatment is very safe and well-tolerated. This data, coupled with positive increases in hair growth efficacy measured at 6-months post-injection, provide support for the Company to move forward with development of a Phase II dosing trial. RepliCel is currently working with European regulators to finalize an application to conduct this Phase II trial, expected to commence in late 2013. This trial will be designed to optimize the regimen utilized for the treatment of AGA through evaluation of several different concentrations of cells and treatment schedules.

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RepliCel Life Sciences is developing a patented, natural hair cell replication technology for the treatment of androgenetic alopecia (AGA) and general hair loss in men and women. This technology is comprised of a patient’s own (autologous) hair follicle cells isolated from the dermal sheath cup (DSC), that have been replicated according to RepliCel’s proprietary cellular replication procedures. The TS001-2009 first-in-man clinical study was designed to test the safety and efficacy of RepliCel’s technology in 10 men and 10 women with AGA. The trial protocol, was developed in compliance with International Conference on Harmonisation guidelines for Good Clinical Practice (ICH GCP) based on advice received from the Paul Ehrlich Institute, an Agency of the German Federal Ministry of Health. Before the study was initiated at the National Scientific Research Centre for Skin and Venereal Diseases in Tbilisi, Georgia, the protocol received a thorough scientific and ethical review by the Georgian National Council of Bioethics which approved the conduct of the study on Oct, 27, 2010.

INTERIM ANALYSES AT 6- AND 12-MONTHS

Once all participants completed their 12-month post-injection follow-up visit, all safety and efficacy data was collected for analysis. Results from this analysis were performed to allow for an accurate assessment of patient safety issues and to provide information to assist in the design of future clinical trials. Additionally, RepliCel analyzed early efficacy at 6-months.

Patient recruitment
• Screened 30 subjects (16 male, 14 female).
• Harvested 25 biopsies (12 male, 13 female).
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Efficacy

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