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</thead>
<tbody>
<tr>
<td>Che Connor</td>
<td>Newcastle University</td>
<td>Scaling up of ambient cell storage, using hydrogel encapsulation.</td>
<td>Building upon previous data to scale up encapsulation of stem cells for storage/transport at room temperature in collaboration with Bioprocessing community.</td>
<td>BBBSRC; EPSRC</td>
<td>Early preclinical</td>
<td>Beginning of 2016</td>
<td>Mesenchymal stem cells, amniotic stem cells (range of stem cells)</td>
<td>-</td>
<td>Allogeneic</td>
<td>No</td>
<td>All</td>
<td>All</td>
</tr>
<tr>
<td>Dr Cedric Ghevaert</td>
<td>University of Cambridge</td>
<td>Platelets derived from induced pluripotent stem cells.</td>
<td>Induced pluripotent stem cell-derived megakaryocytes - new in preclinical studies for platelet production.</td>
<td>MRC; NHPR; Wellcome Trust</td>
<td>Early preclinical</td>
<td>2017</td>
<td>platelets and megakaryocytes</td>
<td>induced pluripotent stem cells</td>
<td>Allogeneic</td>
<td>Yes - combination of lentiviral transduction and genome editing with CRISPR</td>
<td>Haematology</td>
<td>Blood (platelets) for transfusion</td>
</tr>
<tr>
<td>Dr Steve Lee</td>
<td>University of Birmingham</td>
<td>T-cell receptor gene transfer to target Epstein-Barr virus-associated human cancers.</td>
<td>T-cell receptor gene transfer to target Epstein-Barr virus-associated human cancers using T-cell receptors, cloned from CD8+ or CD4+ virus-specific T-cell effectors.</td>
<td>Cancer Research UK</td>
<td>Early preclinical</td>
<td>End of 2015</td>
<td>T-cells</td>
<td>Peripheral blood</td>
<td>Allogeneic</td>
<td>Yes - retrolental gene transfer</td>
<td>Oncology</td>
<td>EBV+ tumours</td>
</tr>
<tr>
<td>Hanns Lochmuller &amp; Chris Bevering</td>
<td>Newcastle University</td>
<td>Induced pluripotent stem cells in Duchenne Muscular Dystrophy.</td>
<td>New in vitro models of Duchenne Muscular dystrophy by induced pluripotency in patient biopsies and gene knockdown in human embryonic stem cells. Ongoing project to investigate the characteristics of dystrophic cardiomyocytes derived from Duchenne Muscular Dystrophy patient biopsies after derivation from induced pluripotent stem cells.</td>
<td>MRC</td>
<td>Early preclinical</td>
<td>End of 2015</td>
<td>induced pluripotent stem cells</td>
<td>-</td>
<td>Allogeneic</td>
<td>No</td>
<td>Cardiovascular</td>
<td>N/A</td>
</tr>
<tr>
<td>Hanns Lochmuller &amp; Jenny Morgan</td>
<td>Newcastle University</td>
<td>Genetically modified stem cells in Duchenne Muscular Dystrophy.</td>
<td>Genetically modified stem cells in Duchenne Muscular Dystrophy. Lentivirally-mediated stem cells to treat Duchenne muscular dystrophy, ongoing project to define the optimal stem cell for repopulating dystrophic muscle on systems application following ex vivo gene correction.</td>
<td>MRC</td>
<td>Early preclinical</td>
<td>End of 2015</td>
<td>CD34+ and/or CD133+ stem cells</td>
<td>-</td>
<td>Allogeneic</td>
<td>Yes - lentiviral transduction</td>
<td>Neurology</td>
<td>Duchenne Muscular Dystrophy</td>
</tr>
<tr>
<td>Majlinda Lako</td>
<td>Newcastle University</td>
<td>Development of synthetic retina</td>
<td>Exploiting the power of human induced pluripotent stem cells to generate synthetic retina in vitro for cell-based therapies, drug discovery and disease modeling.</td>
<td>EU</td>
<td>Early preclinical</td>
<td>-</td>
<td>Retinal ganglion cells</td>
<td>induced pluripotent stem cells</td>
<td>-</td>
<td>No</td>
<td>Ophthalmology</td>
<td>Blindness caused by age-related degeneration of retina or inherited retinal disorders</td>
</tr>
<tr>
<td>Majlinda Lako</td>
<td>Newcastle University</td>
<td>Induced pluripotent cell-based disease model for age-related macular degeneration.</td>
<td>Assessing the feasibility of induced pluripotent stem cells to provide a disease model for age-related macular degeneration.</td>
<td>Other</td>
<td>Early preclinical</td>
<td>-</td>
<td>Retinal pigment epithelial cells</td>
<td>induced pluripotent stem cells</td>
<td>-</td>
<td>No</td>
<td>Ophthalmology</td>
<td>Blindness caused by age-related degeneration of retina</td>
</tr>
<tr>
<td>Majlinda Lako</td>
<td>Newcastle University</td>
<td>PRPF31 patient specific induced pluripotent stem cells.</td>
<td>Improving our understanding of autosomal dominant retinitis pigmentosa, using PRPF31 patient-specific induced pluripotent stem cells.</td>
<td>Other</td>
<td>Early preclinical</td>
<td>-</td>
<td>Retinal ganglion cells</td>
<td>induced pluripotent stem cells</td>
<td>-</td>
<td>No</td>
<td>Ophthalmology</td>
<td>Blindness caused by inherited retinal disorders</td>
</tr>
<tr>
<td>Majlinda Lako</td>
<td>Newcastle University</td>
<td>Stem cells for biological assays of novel drugs and predictive toxicology</td>
<td>This is aimed at deriving human induced pluripotent stem cell line from 500 patients with neurodegenerative disorders.</td>
<td>EU</td>
<td>Early preclinical</td>
<td>2017</td>
<td>Neural progenitor cells</td>
<td>induced pluripotent stem cells</td>
<td>-</td>
<td>No</td>
<td>Neurology</td>
<td>Neurodegeneration</td>
</tr>
<tr>
<td>Prof Mark Loddesell</td>
<td>University College London</td>
<td>Tumour lysis primed natural killer cells for multiple myeloma.</td>
<td>Tumour lysis primed natural killer cells for multiple myeloma.</td>
<td>MRC</td>
<td>Early preclinical</td>
<td>-</td>
<td>Natural killer cells</td>
<td>-</td>
<td>Allogeneic</td>
<td>No</td>
<td>Oncology</td>
<td>Multiple myeloma</td>
</tr>
<tr>
<td>Prof Peter Jones</td>
<td>King's College London</td>
<td>Using mesenchymal stem cells to improve islet transplantation outcome</td>
<td>Co-culturing and co-transplanting islets with mesenchymal stem cells to improve survival and function of islet grafts as a treatment for Type 1 diabetes.</td>
<td>Diabetes UK</td>
<td>Early preclinical</td>
<td>2015</td>
<td>Mesenchymal stem cells</td>
<td>Adipose, bone marrow, pancreas</td>
<td>Allogeneic</td>
<td>No</td>
<td>Diabetes</td>
<td>Type 1 diabetes</td>
</tr>
<tr>
<td>Prof S.B Dunnett</td>
<td>Cardiff University</td>
<td>SMFT</td>
<td>Viability, specificity and yields of clinical grade primary and expanded human fetal cells for neural disorder. Preparation for pilot trial on the safety and feasibility of fetal neural tissue transplantation, including GMP culture protocols.</td>
<td>MRC</td>
<td>Early preclinical</td>
<td>-</td>
<td>Embryonic brain, fetal neural progenitors, human embryonic stem cells and induced pluripotent stem cells</td>
<td>-</td>
<td>Allogeneic</td>
<td>No</td>
<td>Neurology</td>
<td>Parkinson's and Huntington's Disease</td>
</tr>
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## Cell Therapy Catapult Preclinical Database 2015

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<tr>
<td>Yen Choo</td>
<td>Plasticell</td>
<td>Cord blood / hematopoietic stem cells</td>
<td>Adult hematopoietic stem cells expanded ex vivo</td>
<td>Innovate UK</td>
<td>Early preclinical</td>
<td>End of 2015</td>
<td>CD34+ and CD133+ stem cells</td>
<td>Cord blood</td>
<td>Allogeneic</td>
<td>No</td>
<td>Re-population of immune system following chemo/radio therapy</td>
<td>Oncology</td>
</tr>
<tr>
<td>David Choi</td>
<td>UCL</td>
<td>Olfactory ensheathing cells for Brachial Plexus repair</td>
<td>Preclinical development of olfactory ensheathing cells for use in Brachial Plexus repair. Work will include optimisation of cell population and manufacturing process.</td>
<td>-</td>
<td>Early preclinical</td>
<td>-</td>
<td>Olfactory ensheathing cells</td>
<td>Olfactory mucosa of nose</td>
<td>Allogeneic</td>
<td>No</td>
<td></td>
<td>Neurology</td>
</tr>
<tr>
<td>Kevin Docherty</td>
<td>Aberdeen University</td>
<td>IsletCTS</td>
<td>To develop a scalable, cost-effective process for the production of islet cells from pancreatic exocrine tissue.</td>
<td>-</td>
<td>Early preclinical</td>
<td>-</td>
<td>Pancreatic islets</td>
<td>Pancreatic exocrine material</td>
<td>Allogeneic</td>
<td>No</td>
<td>Diabetes</td>
<td>Type 1 diabetes</td>
</tr>
<tr>
<td>Stefano Plachhno</td>
<td>Cambridge University</td>
<td>Induced neural stem cells for progressive Multiple Sclerosis</td>
<td>Development of patient-specific induced neural stem cells as a therapeutic for progressive Multiple Sclerosis.</td>
<td>Multiple Sclerosis Societies; private investment / venture capital</td>
<td>Early preclinical</td>
<td>End of 2016</td>
<td>Directly reprogrammed fibroblasts</td>
<td>Skin- or other accessible sources</td>
<td>Allogeneic</td>
<td>Yes - Sendai virus</td>
<td></td>
<td>Neurology</td>
</tr>
<tr>
<td>Jenny Southgate</td>
<td>York University</td>
<td>Composite Cycloplasty</td>
<td>Development of a novel approach for bladder augmentation utilizing autologous urethral tissue to create a vascularised deconstructed bowel.</td>
<td>MRC</td>
<td>Early preclinical</td>
<td>End of 2015</td>
<td>Urethelial cells</td>
<td>Bladder</td>
<td>Autologous</td>
<td>No</td>
<td></td>
<td>Urology</td>
</tr>
<tr>
<td>Andrew Baker</td>
<td>Glasgow University</td>
<td>Clinical transplantation of endothelial cells derived from human embryonic stem cells.</td>
<td>Generating human endothelial cells from human embryonic stem cell lines under GMP-compliant conditions. Final protocol optimisation and validation, safety studies, efficacy and biodistribution studies.</td>
<td>MRC</td>
<td>Mid preclinical</td>
<td>End of 2016</td>
<td>Endothelial Cells</td>
<td>Human embryonic stem cells</td>
<td>Allogeneic</td>
<td>No</td>
<td>Cardiovascular</td>
<td>Peripheral limb ischaemia</td>
</tr>
<tr>
<td>Dr Maria Serena Longhi</td>
<td>King's College London</td>
<td>Generation and expansion of antigen-specific regulatory T-cells for the treatment of autoimmune hepatitis.</td>
<td>Autologous expanded antigen-specific T regulatory cell population to control effector immune responses.</td>
<td>MRC</td>
<td>Mid preclinical</td>
<td>End of 2015</td>
<td>Regulatory T-cells</td>
<td>Peripheral blood</td>
<td>Autologous</td>
<td>No</td>
<td></td>
<td>Immunology</td>
</tr>
<tr>
<td>Dr Steve Bloor</td>
<td>Viderogen Ltd</td>
<td>Tissue engineered bowel</td>
<td>Develop a tissue engineered bowel for treatment of short bowel syndrome. Utilises decaellularisation/recellularisation technology developed at Northwick Park.</td>
<td>Private investment / venture capital</td>
<td>Mid preclinical</td>
<td>End of 2015</td>
<td>Mesenchymal stem / stromal cells</td>
<td>Bone, bone marrow, adipose tissue</td>
<td>Autologous</td>
<td>No</td>
<td>Gastroenterology</td>
<td>Short bowel syndrome (Crohn’s disease, necrotising enterocolitis)</td>
</tr>
<tr>
<td>Dr Steve Lee</td>
<td>Birmingham University</td>
<td>CLEC14A-targeted T-cells</td>
<td>Genetic modification of T-cells to target the tumour vasculature. Engineering human T-cells to target the tumour vasculature through expression of a Chimeric antigen receptor.</td>
<td>MRC</td>
<td>Mid preclinical</td>
<td>Mid 2015</td>
<td>T-cells</td>
<td>Peripheral blood</td>
<td>Autologous</td>
<td>Yes - CAR gene transfer</td>
<td></td>
<td>Oncology</td>
</tr>
<tr>
<td>Marc Turner</td>
<td>Scottish National Blood Service</td>
<td>Red blood cells derived from pluripotent stem cell lines.</td>
<td>BloodPharma 1 demonstrated that RBCs can be differentiated from human embryonic stem cells and induced pluripotent stem cells, using a feeder and xeno free GMP-grade culture system. BloodPharma 2 aims to optimise the biology and engineering in order to conduct first-in-man clinical study and create a platform for further investment.</td>
<td>Wellcome Trust; Scottish Funding Council</td>
<td>Mid preclinical</td>
<td>2016</td>
<td>Red blood cells</td>
<td>Human embryonic stem cells &amp; induced pluripotent stem cells</td>
<td>Allogeneic</td>
<td>No</td>
<td>Haematology</td>
<td>Beta thalassaemia</td>
</tr>
<tr>
<td>Prof Mark Lowdel</td>
<td>University College London</td>
<td>RegenVox 1</td>
<td>Preclinical development and animal testing of engineered larynx.</td>
<td>MRC</td>
<td>Mid preclinical</td>
<td>-</td>
<td>Mesenchymal stem / stromal cells, epithelium</td>
<td>Bone marrow and tracheal tissues</td>
<td>Autologous</td>
<td>No</td>
<td>Respiratory Medicine</td>
<td>Traumatic injury to larynx</td>
</tr>
<tr>
<td>Prof S.B Dunnett</td>
<td>Cardiff University</td>
<td>Neural progenitor cells from human embryonic stem cells (hESCs) for neurological conditions.</td>
<td>GMP culture protocols for human embryonic stem cell derived neural progenitor cells for Huntington’s disease.</td>
<td>MRC</td>
<td>Mid preclinical</td>
<td>-</td>
<td>Neural stem cells</td>
<td>Human embryonic stem cells &amp; human induced pluripotent stem cells</td>
<td>Allogeneic</td>
<td>No</td>
<td>Neurology</td>
<td>Huntington’s Disease</td>
</tr>
<tr>
<td>Prof. Alan Stitt, Dr Reinhold Medina and Prof Noemi Lois</td>
<td>Queen’s University Belfast</td>
<td>Vascular stem cell therapy for ischaemic retinopathy</td>
<td>Outgrowth endothelial cells from patients with central vein and branch vein ischaemia. Also study of olfactory endothelial cell senescence ex vivo with a view to allowing expansion of a patients cells to allow autologous therapy.</td>
<td>Jules Throm Trust</td>
<td>Mid preclinical</td>
<td>-</td>
<td>Outgrowth endothelial cells / endothelial colony-forming cells</td>
<td>Peripheral blood and cord blood</td>
<td>Autologous</td>
<td>No</td>
<td>Ophthalmology</td>
<td>Ischaemic retinopathies: Central Venous Occlusion &amp; Branch Retinal Vein Occlusion</td>
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<td>Prof. G. Astrid Limb</td>
<td>University College London Institute of Ophthalmology</td>
<td>Preclinical validation of the regenerative potential of retinal ganglion cells derived from Muller stem cells.</td>
<td>Muller stem cells differentiated into retinal ganglion cells are transplanted onto the inner retina of experimental models of retinal ganglion cell damage. Transplanted cells proved to partially restore retinal ganglion cell function in these models.</td>
<td>MRC</td>
<td>Late Preclinical</td>
<td>-</td>
<td>Retinal cells</td>
<td>Retina</td>
<td>Allogeneic</td>
<td>No</td>
<td>Ophthalmology</td>
<td>Glaucoma, retinitis pigmentosa and Age-related macular degeneration</td>
</tr>
<tr>
<td>Sue Kimber</td>
<td>University of Manchester</td>
<td>Pluripotent stem cell-derived Cartilage Cells</td>
<td>-</td>
<td>MRC, ARUK, EU</td>
<td>Mid Preclinical</td>
<td>Late 2015</td>
<td>Chondrocytes</td>
<td>Human embryonic stem cells</td>
<td>Allogeneic</td>
<td>No</td>
<td>Orthopaedics</td>
<td>Osteoarthritis, sports injury and similar conditions</td>
</tr>
<tr>
<td>Mark Kitter</td>
<td>Cambridge University</td>
<td>Olfactory ensheathing cells for Spinal Cord Repair</td>
<td>Optimisation of olfactory ensheathing cells for spinal cord repair to allow a more consistent patient response through definition of the cell population and development of a manufacturing process which produces the same population of cells from each source.</td>
<td>-</td>
<td>Mid Preclinical</td>
<td>-</td>
<td>Olfactory ensheathing cells</td>
<td>Olfactory bulb</td>
<td>Allogeneic</td>
<td>No</td>
<td>Neurology</td>
<td>Spinal cord injury</td>
</tr>
<tr>
<td>Dr. Georgina Ellison</td>
<td>Kings College London</td>
<td>c-kit positive cardiac stem cells in heart failure</td>
<td>c-kit positive cardiac stem cells, resident in the heart, have been demonstrated in animal models to regenerate cardiomyocytes and vasculature. We are now looking to translate this work into the clinical setting in order to perform a first-in-man study for patients with heart failure.</td>
<td>EU</td>
<td>Mid Preclinical</td>
<td>-</td>
<td>c-kit positive Cardiac Stem Cells</td>
<td>Adult myocardiun</td>
<td>Allogeneic</td>
<td>No</td>
<td>Cardiovascular</td>
<td>Heart failure</td>
</tr>
<tr>
<td>Prof. Paolo De Coppi</td>
<td>University College London</td>
<td>Tissue-engineered oesophagus</td>
<td>Development of decellularisation/reconstruction methods for production of a tissue-engineered oesophagus.</td>
<td>UK Stem Cell Foundation</td>
<td>Late Preclinical</td>
<td>End of 2016</td>
<td>Muscle progenitor cells / epithelial progenitor cells</td>
<td>Muscle and oral biopsies</td>
<td>Allogeneic</td>
<td>No</td>
<td>Gastroenterology</td>
<td>Congenital deformity, oesophageal atresia</td>
</tr>
<tr>
<td>Dr Paul Whiting</td>
<td>Pher Neusentis</td>
<td>Retinal pigment epithelium cells</td>
<td>Retinal pigment epithelium cells derived from human embryonic stem cells on membrane.</td>
<td>-</td>
<td>Late Preclinical</td>
<td>-</td>
<td>Retinal pigment epithelium cells</td>
<td>Human embryonic stem cells</td>
<td>Allogeneic</td>
<td>No</td>
<td>Ophthalmology</td>
<td>Age-related macular degeneration</td>
</tr>
<tr>
<td>Prof Sam Jans</td>
<td>University College London</td>
<td>Mesenchymal stem cells - TRAIL</td>
<td>Mesenchymal stem cells genetically engineered to express TNF related apoptosis ligand (TRAIL) as a treatment for lung cancer.</td>
<td>MRC</td>
<td>Late Preclinical</td>
<td>Mid 2017</td>
<td>Mesenchymal stem / stromal cells</td>
<td>-</td>
<td>Allogeneic</td>
<td>Nos engineered to express TNF related apoptosis ligand (TRAIL)</td>
<td>Oncology</td>
<td>Non-small cell lung cancer (adenocarcinoma)</td>
</tr>
<tr>
<td>Dr Steve Bisso</td>
<td>Vidergen Ltd</td>
<td>Tissue engineered autologous stem cell seeded trachea replacement.</td>
<td>Development of a tissue engineered trachea replacement using a decellularized human trachea, seeded with autologous bone marrow derived mesenchymal stem cells and airway epithelial cells. For the treatment of severe structural airway diseases.</td>
<td>Innovative UK private investment / venture capital</td>
<td>Late Preclinical</td>
<td>-</td>
<td>Autologous mesenchymal stem cells and epithelial cells</td>
<td>Bone marrow and airway</td>
<td>Allogeneic</td>
<td>No</td>
<td>Respiratory Medicine</td>
<td>Structural airway diseases</td>
</tr>
<tr>
<td>Prof Madrigal &amp; Dr Saudemont</td>
<td>Anthony Nolan in the UK and Würzburg University in Germany</td>
<td>T-Control Trial</td>
<td>This project aims to evaluate the safety and feasibility of using cord blood regulatory T-cells to treat GvHD in transplanted patients.</td>
<td>EU</td>
<td>Late Preclinical</td>
<td>Beginning of 2015</td>
<td>Regulatory T-cells</td>
<td>Cord blood</td>
<td>Allogeneic</td>
<td>No</td>
<td>Oncology</td>
<td>Chronic Graft vs Host Disease after haematopoietic stem cell transplantation</td>
</tr>
<tr>
<td>Prof S.B Dunnitt</td>
<td>Cardiff</td>
<td>Repair-HD</td>
<td>Preparation for first in man trials on the safety and feasibility of human stem cell-derived trissel tissue transplant in Huntington’s disease.</td>
<td>EU</td>
<td>Late Preclinical</td>
<td>-</td>
<td>Striatal neurons</td>
<td>Clinical grade endothelial stem cell lines</td>
<td>Allogeneic</td>
<td>No</td>
<td>Neurology</td>
<td>Huntington’s Disease</td>
</tr>
<tr>
<td>Bridget Bax</td>
<td>St Georges University London</td>
<td>Clinical development of erythropoietin encapsulated thyminidline phosphorylase a therapy for mitochondrial neurogastrointestinal encephalomyopathy</td>
<td>The project will be conducted in three phases: 1) Validation of processes required for meeting regulatory requirements for operating a clinical trial project; 2) Conducting a multi-centre (pan European), open-label, multiple ascending dose, Phase II trial in 10 patients with mitochondrial neurogastrointestinal encephalomyopathy, over 36 months; and 3) Data analysis and assembly of documentation for regulatory submission.</td>
<td>MRC</td>
<td>Late Preclinical</td>
<td>End of 2016</td>
<td>Red blood cells</td>
<td>Blood</td>
<td>Allogeneic</td>
<td>No</td>
<td>Neurology</td>
<td>Mitochondrial neurogastrointestinal encephalomyopathy</td>
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<tr>
<td>Prof. Giulio Cossu</td>
<td>Manchester University</td>
<td>Pre-clinical development of a stem cell based gene therapy protocol for Duchenne Muscular Dystrophy.</td>
<td>Planning to enhance each step of transplantation (adhesion to and crossing the vessel wall, migration in the muscle ECM, differentiation and enhanced gene correction) through in vitro models. Optimised conditions will be tested in immune deficient dystrophic mice as a proof of principle for developing a new cell mediated gene therapy, optimised protocol for the systemic delivery of autologous, genetically corrected mesangioblasts to Duchenne Muscular Dystrophy patients.</td>
<td>MRC, Biodesign, EC FP7, Duchenne Parent Project.</td>
<td>Late Preclinical</td>
<td>-</td>
<td>Mesangioblasts</td>
<td>Left Extensor Digitorum Brevis</td>
<td>Autologous</td>
<td>Yes - lentiviral transduction</td>
<td>Neurology</td>
<td>Duchenne Muscular Dystrophy Limb Girdle Muscular Dystrophy 2D</td>
</tr>
<tr>
<td>Prof. Paolo Maseddu</td>
<td>Bristol University</td>
<td>Human pericytes for the treatment of ischemia and congenital heart disease.</td>
<td>Pericytes harvested from veins or hearts delivered in models of limb or myocardial ischaemia and in models of congenital heart disease, with standard operating procedure transferred to clinical grade facilities.</td>
<td>British Heart Foundation; MRC, Jules Thorn Trust.</td>
<td>Late Preclinical</td>
<td>-</td>
<td>Pericytes</td>
<td>Vein and Heart</td>
<td>Autologous</td>
<td>No</td>
<td>Cardiovascular</td>
<td>Cardiac repair</td>
</tr>
</tbody>
</table>

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