

Timetable for the ATST#3 Company Presentation

Wednesday, February 1, 2023

#	Time (JST)		Time (Local)			Company	Presenters	Presentation		Country
	Start	End	Start	End	Date			Title	Abstract	
OR	0800	~ 0805	0800	~ 0805		LINK-J	OKANO Hideyuki	Opening Remarks	—	Japan
C-01	0805	~ 0825	1805	~ 1825	-1	Myogenica Inc.	AI HAWKINS	MyoPAXon Stem Cell Therapy for Muscle Disease	Myogenica is a US based startup company developing MyoPAXon, a stem cell platform for regenerating muscle. The therapy repairs muscle fiber and creates new muscle stem cells. Myogenica is beginning a Phase I/II clinical trial in Duchenne muscular dystrophy in 2023.	DE, USA
C-02	0825	~ 0845	1825	~ 1845	-1	Ocugen, Inc.	Arun UPADHYAY, PhD	Modifier Gene Therapy for Retinal Diseases	Retinal degenerative diseases can be broadly categorized into inherited genetic disorders – such as retinitis pigmentosa, leber congenital amaurosis; and complex multifactorial diseases – for example age related macular degeneration. Mutations in more than 300 genes have been identified to cause inherited retinal disorders and this number keeps increasing with time as more genetic screening are performed. In last decade, gene therapy has shown immense potential and offered promise for potential treatment of these genetic diseases. However, because of mutational diversity and multitude, development a single product of each gene mutation is significantly slower due to development cost, technical challenges, timeline and limited commercial opportunity. One way to address these conditions are to develop a therapeutic which has potential to improve retina health, function, and survival – irrespective of disease etiology. Modifier genes, a class of nuclear hormone receptor-based transcription factors, offer such potential and have demonstrated ability to regulate multiple molecular pathways and restored cellular homeostasis in retina. Ocugen, Inc. is developing two products based on modifier gene therapy technology: (1) OCU400 (NR2E3 gene) – which is currently in phase1/2 clinical development for the treatment of RP and LCA diseases and (2) OCU410 and OCU410ST (both based on RORA gene) for the treatment of dry age related macular degeneration and Stargardt diseases.	PA, USA
C-03	0845	~ 0905	1845	~ 1905	-1	Progenicyte Japan Co., Ltd.	SUGAYA, Kiminobu, PhD	Exosomes as the nucleic acid medicine delivery system	Exosome naturally produced by the cells contains cytosolic information, which is safe for DDS. Furthermore, the modifications of the surface membrane allow us to target specific tissue and cells. Here, we developed a technology to efficiently introduce DNA into exosomes while the host cells produce them. We will show applications of this technology in cancer and COVID-19 therapies.	FL, USA (Japan)
BR	0905	~ 0915	~			Break				
C-04	0915	~ 0935	1915	~ 1935	-1	SparingVision SAS	Daniel CHUNG, DO, MA Raffaella TOSO, PhD	SparingVision's gene-agnostic approach for ocular diseases	SparingVision is focused on genomic medicines for ophthalmic diseases. SparingVision's lead asset SPVN06 is a breakthrough gene therapy approach aimed at stopping or slowing disease progression in patients affected by Inherited Retinal Diseases (IRDs) and dry age-related macular degeneration (AMD), regardless of their genetic background. FDA cleared the IND application for SPVN06. This approval paves the way for the initiation of Promising Rod-cone Dystrophy Gene therapy (PRODYG), a first-in-human (FIH) Phase I/II clinical trial. First safety data are anticipated in 2023 and the primary endpoint is expected to be reached in 2025.	PA, USA (France)
C-05	0935	~ 0955	1835	~ 1855	-1	Ronawk, Inc.	A.J. MELLOTT, PhD	Leveraging A Modular Hydrogel to Mass Produce Stem Cells for Therapeutic Applications	Bio-Blocks are interconnecting hydrogel blocks that can be tuned for accommodating specific cell types or different applications. Each Bio-Block is permeated with microchannels that run through the entire Bio-Blocks. When two Bio-Blocks are connected, the microchannels between the two Bio-Blocks align to make a continuous network in which stem cells may proliferate and migrate throughout both Bio-Blocks. Stem cells grow throughout the microchannels uninterrupted. Bio-Blocks may be arranged in plates and used in tandem with a liquid handling system for large-scale cell production. Bio-Blocks simplify the cell culture process by brining uniformity, consistency, and repeatability while reducing contamination risk from human involvement and reduce media consumption. Furthermore, Bio-Blocks maintain stem cell phenotypes longer than traditional cell culture methodologies while protecting stem cells against senescence.	KS, USA
C-06	0955	~ 1015	1755	~ 1815	-1	RheumaGen, Inc.	Brian FREED, PhD, MA, MS	Introduction to RheumaGen: Neutralizing the genetic "trigger" for Rheumatoid Arthritis	RheumaGen is developing a gene-editing platform for the HLA molecule that is invisible to the human immune system, allowing us to use an infusion of gene-edited autologous hematopoietic stem cells to reprogram the immune system and potentially cure rheumatoid arthritis and other autoimmune diseases without broad immunosuppression. RheumaGen's initial market is the approximately 10% of rheumatoid arthritis patients who cannot benefit from currently available treatments. The technology was discovered by an experienced team at ClinImmune Labs at the University of Colorado and RheumaGen partners with them to continue the technology's development. The RheumaGen therapy candidate for rheumatoid arthritis is in the late preclinical stage, and we are preparing an IND for submission to the FDA with plans to begin Phase 1 clinical trials within the next 12-18 months.	CO, USA
C-07	1015	~ 1035	1715	~ 1735	-1	Immusoft Corp.	Sean AINSWORTH	tba	tba	WA, USA
BR	1035	~ 1045	~			Break				
C-08	1045	~ 1105	1045	~ 1105		ThinkCyte Inc.	MIZUTANI, Mirai, PhD	Development of Ghost Cytometry applications - AI-driven cell sorting technology based on cell morphology -	This technology is a new type of cell discrimination and sorting technology that integrates advanced technologies in optics, machine learning, and microfluidics. We have developed a label-free measurement, which enables us to discriminate the phenotypic differences of target cells. It is expected to become an epoch-making tool in the healthcare field where cells are handled, such as in the manufacturing process of cell therapy products, in the sample pretreatment and observation process of clinical tests such as blood samples, and in the search for target molecules in drug discovery research.	Japan (CA, USA)
C-09	1105	~ 1125	1105	~ 1125		Celaid Therapeutics, Inc.	Dean MERKEL	Providing a Better Alternative to Stem Cell Transplantation	Celaid Therapeutics is developing cord blood-derived allogeneic hematopoietic stem cell therapy products as an alternative to current stem cell transplantation. We aim to become the new standard for treatment of, and even cure for, hematological diseases.	Japan
C-10	1125	~ 1145	1125	~ 1145		Delta-Fly Pharma, Inc.	ESHIMA, Kiyoshi, PhD	Module Drug Development	Module Drug Development is the assembly of various active ingredients into novel anticancer agents. With this approach and application of drug delivery technologies and knowledge about dosage and administration, drugs with a good balance of safety and efficacy can be developed in a short period of time.	Japan
L-01	1145	~ 1205	~			Alliance for Regenerative Medicine	Tim HUNT, CEO	State of the Cell and Gene Therapy Industry in 2023 (Pre-recorded)	HighHighlight of key industry trends and anticipated product approvals in 2023.	DC, USA
BR	1205	~ 1305	~			Break				
C-11	1305	~ 1325	1305	~ 1325		iHeart Japan Corp.	JIANG, Zixian	iPSC-Based Regenerative Medicine for Heart Failure	iHeart Japan Corporation is developing iPSC-derived multilayered cardiac cell sheets to treat severe heart failure. We aim to realize regenerative medicine for heart failure and release patients from the heart transplant waiting list.	Japan
C-12	1325	~ 1345	1325	~ 1345		iXgene Inc.	MIZUNO, Atsushi, PhD	Suicide gene therapy and regenerative medicine using genome-edited iPS cells	tba	Japan

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C-13	1345	~ 1405	1345	~ 1405		Luxna Biotech Co., Ltd.	SATO, Hideaki, MSc	Promising platform technologies for nucleic acid drug discovery and current drug development progress	Luxna Biotech is a preclinical stage antisense drug development company. Our core technologies are novel modified nucleosides called "XNAs" invented by Osaka University. Today, we'd like to talk about our promising platform technologies for antisense drug discovery and current drug development progress.	Japan
C-14	1405	~ 1425	1405	~ 1425		Minaris Regenerative Medicine Co., Ltd.	NAKASHIMA, Katsuhiko, PhD	CGT CDMO with bases in Japan, the U.S., and Europe, introduces large-scale cell manufacturing using 3D bioreactor	Minaris Regenerative Medicine is a global contract development and manufacturing organization (CDMO) for cell and gene therapies (regenerative medicine). We offer our clients clinical and commercial manufacturing services, development solutions, and technologies. In this seminar, we would like to explain our view points for establishing an organization for commercial manufacturing of regenerative medicine and 3D bioreactor platform for large scale manufacturing.	Japan
BR	1425	~ 1435	~			Break				
C-15	1435	~ 1455	1435	~ 1455		PeptiGrowth Inc.	SUGIMOTO, Jiro	Synthetic peptide-based growth factors "Forging the Path Forward for Cell Therapy and Regenerative Medicine"	To Forge the path forward for cell therapy and regenerative medicine, PeptiGrowth is developing novel synthetic peptide-based growth factors which have equivalent function to conventional recombinant growth factors. We will introduce our technologies, current and upcoming products in this presentation.	Japan
C-16	1455	~ 1515	1455	~ 1515		PuREC Co., Ltd.	MORISHITA, Yoshikazu, PhD	Bone & Cartilage Regeneration with New Cell Therapy "REC"	PuREC is a stem cell company with its proprietary technology to provide the extremely purified stem cell, "REC". "REC" is currently used in two investigator-led clinical trials: 1) Ph-I/II a for Hypophosphatasia at Shimane University, and 2) Ph-I/II a for Lumber / Spinal Canal Stenosis at Hokkaido University.	Japan
C-17	1515	~ 1535	1145	~ 1205		Stempeutics Research Pvt. Ltd.	Dr Pawan Kumar GUPTA, MD, DNB, PhD, MNAMS	Allogeneic, Bone Marrow derived, Pooled, Mesenchymal Stromal Cells – A Potential Break through Therapy for a) Grade II & III Osteoarthritis Knee Management b) Critical Limb Ischemia due to Buerger's disease	Osteoarthritis of knee joint Phase 3 data presentation: 146 patients of primary osteoarthritis having Grade II & III OA based on Kellgren and Lawrence radiographic criteria were randomized to stem cell and placebo group in a ratio of 1:1. 73 patients each received either a single intra-articular injection of stempeucel® (25 million cells) followed by 20mg hyaluronan or Placebo followed by 20mg hyaluronan under ultrasound guidance. The primary end point was evaluation of WOMAC Composite Index score as compared to the placebo arm. Secondary end points were – WOMAC sub-scores: pain, stiffness & physical function, VAS & MRI assessment for T2 mapping, volume & thickness. WOMAC composite index, the primary end point of the study, showed significant improvements in cell arm as compared to placebo at month 6 and month 12 (mean difference: -632.74; 95% CI [-791.57 - -473.91], P<0.0001; percentage change: -44.3%). Additionally stempeucel® significantly improved WOMAC pain, stiffness, physical function and VAS scores at 6 and 12 months (P<0.0001 {for all parameters}). T2 mapping showed no worsening of the deep cartilage in the medial femoral tibial compartment of the knee in stempeucel® arm at month 12 follow up within the group whereas in placebo arm there is significant and gradual worsening of the cartilage seen within the group at month 12. Cartilage volume shows there is increase in average cartilage total volume of 34.07 units in stempeucel® arm irrespective of time. Based on Phase 3 data, received marketing authorization from Indian FDA (DCGI). Stempeucel® has proven to be safe and effective for treatment of Grade II and Grade III Osteoarthritis of knee. The intervention is simple, easy to administer, does not require surgery, provides sustained relief of pain, stiffness, improves physical function, prevents worsening of cartilage quality and improves cartilage volume for at least 12 months follow up. Critical Limb Ischemia (CLI) due to Buerger's disease Total of 160 patients of CLI due to Buerger's disease were exposed to stempeucel® in all clinical trials of "no – option" patients of CLI in Rutherford classification - III-5 and Rutherford- III 6 (with gangrene limited to the toes) who are not eligible for revascularization. The efficacy in the clinical study was measured by two primary end points - relief of rest pain and healing of ulcer. Initially a dose finding study was done in two doses of - 1 and 2 million cells/kg body weight and was compared with standard of care arm. The dose of 2 million / kg was found to be most efficacious. Thereafter, in all the clinical trials the dose of 2 million/kg was used. In the phase 2 and phase 4 clinical trials, both the primary efficacy end points were statistically significant (p<0.05). The secondary efficacy end points of increase in Ankle Brachial Pressure Index, in ankle systolic pressure, in total walking distance and quality of life were also statistically significant (p<0.05). In the phase 2 study, the MRA showed evidence of increase collaterals in the cell arm. Hence, IM administration of Stempeucel® at a dose of 2 million/kg group has shown clinical benefit and is considered to be effective in treating patients of CLI due to Buerger's disease. Stempeutics had been granted manufacturing & marketing approval for both the above indication by Indian FDA. We are looking for strategic partner to globalize Stempeucel® product for the treatment of Osteoarthritis of knee joint and Critical Limb Ischemia due to Buerger's disease.	India
BR	1535	~ 1545	~			Break				
C-18	1545	~ 1605	0745	~ 0805		eTheRNA Immunotherapies NV	Tim VAN ASSCHE, PhD	TriMix and tumor antigen mRNA electroporated dendritic cell vaccination to treat melanoma and gastric cancer	eTheRNA is a developer of mRNA-based technologies, with in-house GMP manufacturing capabilities and proprietary platform technologies. We have a clinical-stage melanoma or gastric cancer immunotherapy which is now available for partnering. Our novel immunotherapy consists of autologous dendritic cells (DC) transfected ex vivo with eTheRNA's proprietary TriMix-mRNA immune stimulant, together with mRNA's encoding clinically validated melanoma or gastric cancer associated antigens. This ex vivo approach has been shown to be safe & well tolerated, and efficacy with this approach has already been clinically demonstrated by eTheRNA in multiple Phase 2 studies in melanoma patients. In these studies, we demonstrated a clear link between the induced antigen-specific T-cell immunity and clinical response, including significantly improved 3-year survival and overall response rates.	Belgium
C-19	1605	~ 1625	0805	~ 0825		PDC*line pharma SA	Eric HALIOUA, MBA. MSc.	New class of cancer vaccine based on an off-the-shelf Antigen Presenting Cell line (PDC*line)	• PDC*line is a new potent and scalable therapeutic cancer vaccines based on a proprietary allogeneic cell line of Plasmacytoid Dendritic Cells • PDC*line is much more potent to prime and boost antitumor antigen, including neoantigens, specific cytotoxic T-cells than conventional vaccines and improves the response to checkpoint inhibitors • Presentation of first immuno and clinical results of our ongoing NSCLC clinical trial	Belgium

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C-20	1625	~ 1645	0825	~ 0845		EG 427	Philippe CHAMBON, MD PhD	Non-replicative Herpes Simplex Vectors for next generation gene therapies	In vivo gene therapies have reached market but also faced the limits of current viral vectors. EG 427 develops next generation gene therapies to overcome these limits. With high payload capacity, long term expression, demonstrated safety profile and efficient manufacturing process, our vectors can address local chronic diseases in the nervous system and beyond.	France
C-21	1645	~ 1705	0845	~ 0905		Eligo Bioscience SAS	Aurelie GRIENENBERGER, PhD	Eligo Bioscience: improving patients' lives through precision gene editing of the microbiome	Eligo is pioneering gene editing to the microbiome to durably remove bacterial drivers of disease with unprecedented precision. Eligobiotics is a new modality to precisely edit deleterious bacterial genes from disease-driving microbiota. Eligobiotics are delivery vectors derived from bacteriophage that can package a DNA payload, equipped either with a CRISPR nuclease to efficiently remove a deleterious gene by killing the bacteria that carry it, or with a base editor to modify the genetic code of a deleterious gene, for instance to inactivate it, without killing the bacteria expressing it. Eligo is advancing a highly differentiated pipeline of precision gene editing medicines to address unmet medical needs in inflammation, autoimmunity and oncology caused by the expression of specific deleterious bacterial genes from our microbiome.	France
BR	1705	~ 1715		~		Break				
L-02	1715	~ 1735	0815	~ 0835		4Bio Capital	Owen SMITH	tba	tba	UK
C-22	1735	~ 1755	0935	~ 0955		TreeFrog Therapeutics SAS	Maxime FEYEUX, PhD	Scaling-up iPSC-based cell therapies: real-world processes with biomimetic C-Stem technology	For the past 10 years, developmental biologists have explored novel approaches to mimic the in vivo niche and 3D architecture of human pluripotent stem cells (hPSCs). Taniguchi et al. (2015, 2017) demonstrated that, in permissive in vitro conditions, PSC colonies spontaneously self-organize and polarize in 3D around a central lumen. Furthermore, this in vivo-like "lumenized rosette" conformation was recently shown to promote PSC proliferation, homogeneous pluripotency, and maintenance of genomic integrity [Kim et al. 2021; Knouse et al., 2018; Hashimoto et al., 2019]. Based on high-speed cell encapsulation microfluidics, the C-Stem technology enables mass-producing 3D hiPSC lumenized rosettes protected within an alginate shell. In 2021, C-Stem allowed unprecedented 276-fold expansion of hiPSC within 7 days in 10L bioreactors, yielding single batches of 15 billion iPSCs with best-in-class quality. Following the delivery of a GMP-compliant C-Stem platform, our presentation aims to show C-Stem-based processes for the expansion and differentiation of iPSC into neural microtissues for the treatment of Parkinson's disease.	France
C-23	1755	~ 1815	0955	~ 1015		Salipro Biotech AB	Maria KNUDSEN, PhD	Salipro One-Step Reconstitution of Challenging Membrane Protein Targets for Drug Development	Salipro Biotech's proprietary nano-membrane technology (Salipro®) stabilizes challenging drug targets. With Salipro® it is possible to develop therapeutic antibodies or small molecules targeting difficult membrane proteins (GPCRs, ion channels, SLC transporters, etc.). Salipro Biotech has signed multiple research collaborations with top-tier pharma and biotech companies (e.g. Sanofi, AstraZeneca, AbCellera) in the context of the Salipro® platform. In addition, Salipro Biotech is pursuing an internal pipeline program.	Sweden
C-24	1815	~ 1835	1015	~ 1035		Verigraft AB	Petter BJORQUIST, PhD	Personalized tissue-engineered grafts that will revolutionize future medicine	VERIGRAFT AB is a Swedish biotechnology company run by experienced entrepreneurs and mastering a unique, breakthrough technology in the field of advanced therapies and regenerative medicine. We make transplantation possible without the severe risks of immunosuppression, and develop advanced therapies and tissue engineered products that will be able to help millions of patients with serious diseases.	Sweden
BR	1835	~ 1845		~		Break				
C-25	1845	~ 1905	1045	~ 1105		Xintela AB	Evy LUNDGREN-AKERLUND	tba	tba	Sweden
C-26	1905	~ 1925	1105	~ 1125		Orchard Therapeutics plc	Jeffery S. VICK, MS, MBA	Orchard Therapeutic's Hematopoietic Stem Cell Therapy	Orchard Therapeutics' vision is to end the devastation caused by genetic and other severe diseases using the curative potential of hematopoietic stem cell (HSC) gene therapy. In this approach, a patient's own blood stem cells are genetically modified outside of the body and then reinserted, with the goal of correcting the underlying cause of disease in a single treatment. Our focus is on rare diseases and neurometabolic disorders. Libmeldy, indicated for the treatment of MLD, is approved and marketed in Europe and the US BLA will be filed early 2023. Our program in MPS-I has compelling clinical data and we expect to commence a registrational study in 2023. We have additional programs in MPSII and MPSIII, Crohn's disease, HAE and FTD, as well as Tregs for the treatment of Multiple Sclerosis and other autoimmune diseases. Orchard seeks partners to help us advance these programs.	Switzerland (UK)
C-27	1925	~ 1945	1025	~ 1045		Laverock Therapeutics Ltd.	Tim ALLSOPP, PhD	Utilizing Gene Editing Induced Gene Silencing to unlock the next generation of programmable cell therapies	Laverock Therapeutics has a proprietary platform for inducing the silencing of new target genes in therapeutic cells and by this approach can instil novel functionality and enhanced performance of the therapy. Gene Editing induced Gene Silencing (GEIGS®) utilizes the cells' own endogenous microRNA expression profiles and redirects them to knock down target genes of choice. By introducing the cognate sequence changes for the miRNA at the DNA-level, this generates silencing in a stable, highly consistent, tunable and programmable manner. As the physiological pattern of recoded miRNA expression remains unchanged as the minor edits are introduced and therefore linked to cell identity, the new target gene silencing occurs in response to the stage of cell development, its' activation, or its' responsiveness to an in vivo disease related environment. The novel effect can be pre-programmed in edited cells and the conditionality of silencing is a feature that is unique to GEIGS and not possible to obtain with other conventional RNAi approaches. Laverock is developing the technology to achieve programmed polarisation of macrophages as a therapeutic concept targeting the immunosuppressive tumour micro-environment for hard-to-treat cancers, and conditional hypoinmunogenicity of stem cell-derived islets as a cell replacement therapy in Type 1 diabetes. It is also interested in exploring options for partnering for other indications.	UK
C-28	1945	~ 2005	1045	~ 1105		Touchlight DNA Services Ltd.	Rhona McIntyre	tba	tba	UK
CR	2005	~ 2010	2005	~ 2010		LINK-J	MORI, Fuyuhiko	Acknowledgments	—	Japan

OR	Opening Remarks
L-xx	Invited Lecture

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	Start	End	Start	End	Date			Title	Abstract	
C-xx	Company Presentation									
BR	Break									
CR	Closing Remarks									