Drug Candidates

GEMSEKI

As of 03/25/20

Date	Candidate	Mechanism of action	Indication	Route	Modality	Disease area	Development stage	Note
03/25/20	GEM035	An anti-ENO1 antibody	Immune diseases, Various cancers	s.c.	Protein	Cardiology CNS Gastroenterology Immunology/ Inflammation Oncology: Solid cancer Respiratory Rheumatology	US FDA IND cleared	GEM035 is a humanized antibody against unique ENO1 target. This therapeutic antibody is first-in-class with macrophage-targeting features and showed efficacy in animal model for MS, IPF, and IBD. It also showed efficacy in animal model for liver cancer, pancreatic cancer, and lung cancer. GEM035 has a worldwide patent protection. US IND of GEM035 is approved and active now for treating MS.
03/13/20	GEM165	Intratumoral production of 5-FU and reduction of T-reg in the tumor	Colorectal cancer, Esophageal cancer, Gastric cancer	i.v.	Bacteria	Oncology: Solid cancer	Phase 2 ready	GEM165 is recombinant Bifidobacterium modified to express cytosine deaminase (CD). Bifidobacterium is anaerobic bacteria constituting human intestinal flora. Intravenously administered GEM165 colonizes to hypoxic solid tumor specifically and converts orally administered 5-FC to a high concentration of 5-FU only inside the tumor. The preclinical data of GEM165 in combination with anti-PD-1 antibody demonstrate synergistic anti-tumor efficacy accompanied by drastic Treg reduction. Note: ・First-in-class, patented, strong preclinical data of GEM165 alone and in combination with PD-1 blocker. ・Clinical experiences in over 30 patients in the Phase 1 study in the US. ・The design of Phase 2 combination therapy with anti-PD-1 antibody has been already consulted with FDA. The study is ready to start after completion of the Phase 1 study.
03/11/20	GEM164	Anthracycline topoisomerase II inhibitor	Breast cancer, Bladder cancer, Kaposi's sarcoma, lymphoma, and Acute lymphocytic leukemia	i.v.	Small molecule	Oncology: Hematological cancer Oncology: Solid cancer	Bioequivalence study completed	Generic pegylated liposomal doxorubicin hydrochloride. Doxorubicin is well known to cause cardiotoxicity and develop congestive heart failure. Cardiotoxicity of GEM164 is expected to be substantially lower than non-liposomal doxorubicin. Bioequivalence with CAELYX has been demonstrated.
03/02/20	GEM163	Reduction of virus-replication and reduction of inflammation	Virus diseases (incl. coronaviruses)**	Oral	Oligo-saccharides	Infection	NDIN** ready	A novel, intestinally absorbable derivative (pat. pend.) of GRAS αCD (α-cyclodextrin) to reduce virus entry (endocytosis) and replication/assembly of virueses (availability of lyso-phospholipids). βCDs have been effective in vitro against many virus infections, incl. coronaviruses, and topically against influenza and HSV2. αCDs avoid the ototoxicity of βCDs and were more effective (tested in HIV-1 cells). **New dietary ingredient notification as a nutritional supplement/FSMP
03/02/20	GEM162	Restoration of autophagy and reduction of inflammation	Age-related diseases**	Oral	Oligo-saccharides	Others	NDIN** ready	A novel, intestinally absorbable derivative (pat. pend.) of GRAS αCD (α-cyclodextrin) as an intermittent fasting mimetic. βCDs have been effective in vivo against many age-related diseases, including cancer, AD, and PD. αCDs are more effective against endocytosis than β CDs and lack the βCDs' ototoxicity. In the EU, oral αCD may claim to "reduce post-prandial glycemic response", but has low and variable bioavailability. **New dietary ingredient notification as a nutritional supplement/FSMP
03/02/20	GEM161	Restoration of autophagy and reduction of inflammation	Cardiovascular and Metabolic Diseases	Oral	Oligo-saccharides	Cardiology Metabolic disease	Phase 2b/3 ready	A novel derivative (pat. pend.) of hydroxypropyl-α-cyclodextrin (HPαCD) that down-regulates the PI-system by controlling serum phospholipids and, thereby, reduces endocytosis. βCDs have been shown to be effective in vivo against atherosclerosis (AS), NAFLD, but can cause permanent hearing loss (not applicable to αCDs). Oral αCD is clinically effective against metabolic syndrom, but has low and variable bioavailability. 505(b)(2) is applicable.

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03/02/20	GEM160	Restoration of autophagy and reduction of inflammation	Neuro-degenerative diseases	Oral, Intra-thecal	Oligo-saccharides	CNS	Phase 2b/3 ready	A novel derivative (pat. pend.) of hydroxypropyl-α-cyclodextrin (HPαCD) that down-regulates serum phospholipids and prevents aging cells from accumulating Aβ/tau (AD), α-syn (PD), myelin (MS), mHTT (HD), SOD1 (ALS), ... βCDs were effective in vivo against AD and PD, but development was abandoned (except for NPC) due to the risk of permanent hearing loss (not applicable to αCDs). In the US, αCD is generaly recognized as safe (GRAS) for oral use; in the EU, αCDs are approved for oral and parenteral use. 505(b)(2) is applicable.
03/02/20	GEM159	Restoration of autophagy and reduction of inflammation	Breast cancer and other carcinomas*	Oral, intra-venous	Oligo-saccharides	Oncology: Solid cancer	Phase 2b/3 ready	A novel intestinally absolrbable derivative (pat. pend.) of hydroxypropyl-α-cyclodextrin (HPα CD) that down-regulates the Phosphoinositide-system by controlling serum phospholipids and, thereby, reduces endocytosis. βCDs were effective in vivo against breast, ovarian, lung, and colon cancer, and metastatic melanoma, but need to be infused overnight and can cause permanent hearing loss. αCDs are not ototoxic and were more effective in vivo against growth and metastases of breast cancer. *Mono- or adjuvant treatment. 505(b)(2) is applicable.
02/27/20	GEM158	Anti-mitotic chemotherapy	Small cell lung cancer	i.v.	Small molecule	Oncology: Solid cancer	Phase 1 completed	Proprietary innovative albumin-stabilized pegylated liposomal docetaxel formulation. Elevated exposures of docetaxel compared with free (nonencapsulated) docetaxel were confirmed in animals and humans. Acceptable tolerability and results suggesting anti-tumor efficacy were observed in Phase 1. FDA orphan drug designation was granted and confirmed with FDA that 505(b)(2) NDA pathway appears to be an acceptable approach.
02/21/20	GEM157	Combined adoptive cell therapy (autologous)	Hepatocellular carcinoma (HCC)*	Infusion	Cell therapy	Oncology: Solid cancer	Launch	A combined adoptive cell therapy comprising cytokine-induced killer cells and activated cytotoxic T lymphocytes. In Phase III using patients whose tumors have been removed after curative resection for HCC, RFS was 44 months for the immunotherapy group while that of the control group was 30 months. The HR for tumor recurrence or death in the immunotherapy group vs the control group was 0.63. The mortality rate was reduced by 79% in the immunotherapy group vs the control group. Clinical trials for other solid tumors are ongoing. *: Adjuvant therapy for patients whose tumors have been removed after curative resection for HCC.
02/18/20	GEM156	Chromatin destabilizing	Solid and hematological tumors	Oral, i.v., i.a	Small molecule	Oncology: Hematological cancer Oncology: Solid cancer	Phase 1	A First-In-Class chromatin destabilizing agent that intercalates into DNA, and interferes with histone/DNA binding changing its spatial structure. Consequent functional inactivation of a histone chaperon FACT leads to inhibition of several previously undruggable pro-cancer transcriptional factors, activation of p53 and interferon response. Dose-dependent nonclinical antitumor activity is seen in multiple models of solid and hematological tumors. Oral and i.v. phase 1 studies demonstrated a manageable safety profile and disease control with tumor regressions and protracted stable disease.
02/18/20	GEM155	FPR2-specific ligand	Atopic dermatitis/Psoriasis, Dry eye disease, IBD (Inflammatory bowel disease), Asthma, Rheumatoid arthritis	Topical, Eye drop, s.c.	Peptide	Dermatosis Immunology/ Inflamation Ophthalmology Rheumatology	Preclinical	GEM155 is a small (7mer) lipidated peptide ligand for pro-resolving receptor FPR2 (N-formyl peptide receptor 2) involved in regulation of innate immune system and inhibition of ILC2 function (adaptive immune system). It also has anti-microbial effect for pathogenic bacteria through fusion with functional moiety. Efficacy is seen in animal models for the indications. CMC study is almost done. Toxicity study for topical usage and subcutaneous injection is going-on. Formulation for topical use is almost finished.
02/18/20	GEM154	Collagen-inducing peptide	Dermal filler, Cosmetics	Topical	Peptide	Dermatosis Others	Preclinical	Laminin-derived peptide. Boosts collagen synthesis at sub-nanomolar concentration (Wrinkle-care) & inhibits melanin synthesis (Whitening). Registered cosmetic ingredient.
02/18/20	GEM153	Angiogenic peptide	Wound-care, Diabetic foot ulcer, Cosmetics	Topical	Peptide	Dermatosis Metabolic disease Others	Preclinical	Increases blood vessel formation (VEGFA/VEGFR1 expression ↑& cell proliferation/migration ↑). Boosts collagen synthesis at sub-nanomolar concentration (Wrinkle-care) & inhibits melanin synthesis (Whitening). Registered cosmetic ingredient.

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02/18/20	GEM152	Fat-adsorption inhibitor	Obesity, Diabetes, Fatty liver	Oral	Natural product	Metabolic disease	Preclinical	Mushroom-derived natural product. Reduces weight gain and obesity, blood glucose and lipid contents in the liver via reduction of lipid absorption in the gut.
02/18/20	GEM151	anti-GM-CSF monoclonal antibody	Rheumatoid arthritis & multiple new indications* (see note)	i.v.	Antibody	Immunology/ Inflammation Rare disease Rheumatology	Preclinical (ready for IND-enabling studies)	GM-CSF is a key player in inflammation and autoimmunity. GEM151 is a fully human monoclonal antibody generated by single B cell cloning and has superior affinity (Kd: 7.3 X 10 -11 M) compared to competitors. Neutralizing activities were confirmed by four different functional assays. *cytokine release syndrome., GvHD, multiple sclerosis/neuroinflammation, Kawasaki disease
02/12/20	GEM150	Melanocortin 4 & 5 agonist	Obesity	Oral	Peptide	Metabolic disease	Preclinical	A pro-drug of the cyclic peptide (BL3020-1) which is a melanocortin 4 & 5 agonist showing good permeability in the gut and BBB and great reduction in food consumption and body weight gain in mice. The pro-drug did not show aggregation which BL3020-1 did. Currently the pro-drug is being optimized.
01/29/20	GEM149	PKC modulator	Alzheimer disease	Oral, Intranasal	Small molecule	CNS	Phase 1	Up-regulates production of α-secretase which cleaves the amyloid precursor protein, APP, into a harmless soluble form, sAPP-α, which is non-neurotoxic and limits the formation of amyloid plaques.
01/29/20	GEM148	CCR5 inhibition	HIV	i.v.	Nucleic acid	Infection	Preclinical	GEM148 is a nanoencapsulation of proprietary, synthetic CCR5-siRNA targeted to knock-down and eliminate HIV-1 in chronic HIV patients as well as prevent HIV-1 infection in naïve patients and re-infection in acutely-infected patients.
01/29/20	GEM147	Insulin	Diabetes	Oral	Peptide	Metabolic disease	Preclinical	GEM147 is a nanoencapsulation of insulin in biodegradable polymer nanospheres. Nanoencapsulation protects insulin during stomach passage. In vivo, statistically significant reduction in blood glucose was seen in diabetic rats with oral GEM147 within 30 minutes.
01/29/20	GEM146	VD Receptor	Prostate cancer, Autoimmune diseases	i.v.	Small molecule	Immunology/ Inflammation Oncology: Solid cancer	Preclinical	GEM146 is a nanosomal formulation of AMPI-109, a nontoxic Vitamin D3 analog. In vivo studies have shown strong anticancer effects of GEM146 against Hormone Refractory Prostate Cancer xenografts in nude mice at doses approximately 6.5 times less than the parent hormone, without significant toxicity.
01/29/20	GEM145	5-HT	Chemotherapy Induced Nausea and Vomiting (CINV)	Oral	Small molecule	Oncology: Supportive care	Phase 2/3	Acute vomiting was blocked effectively by 5-HT3 anti-emetics plus the adjuvant Emend® but no benefits were observed in terms of the incidence of nausea . Phase 2/3 clinical trial on GEM145 as an adjuvant to conventional 5-HT3 anti-emetics was completed (more than 600 cancer patients). All doses of GEM145 significantly reduced acute nausea severity compared to the placebo (p=0.003).
01/29/20	GEM144	PKC modulator plus HDAC inhibitor	HIV Latency	i.v., Oral	Small molecule	Infection	Phase 2a	GEM144 is a nanoencapsulation of PKC modulators plus HDAC inhibitor in targeted, pegylated phospholipid nanosomes for improved therapeutic index. Activates HIV from latent reservoirs so that HIV can be eradicated from the body by antiviral therapy and/or immune system.
01/29/20	GEM143	CB1, CB2, 5-HT1a	Multiple Sclerosis (MS), Opioid addiction, Chemotherapy induced Peripheral Neuropathic Pain (CIPNP), and Epilepsy	Oral	Small molecule	CNS Immunology/ Inflammation Oncology: Supportive care Pain/Neuropathy Rare disease	Preclinical	GEM143 is a nanoencapsulation of the API in biodegradable polymer nanospheres for improved oral bioavailability. MS: The API has multiple actions on key molecular neuroinflammation and neurodegeneration targets, with highly effective in vitro and in vivo bioactivities. Opioid addiction: The API is not an opioid and has been shown to alleviate cue-induced opioid addiction behaviors and allosterically modulate the μ and δ-opioid receptors. CIPNP: Nanoencapsulation is being used to protect the API during the stomach passage. Epilepsy: The API is an effective anti-convulsant with a specificity more comparable to drugs clinically effective in major seizures.

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01/20/20	GEM142	Nrf2 Activator	COPD and Diabetic kidney disease	Oral	Small molecule	Metabolic disease Respiratory Urology	Phase 1	A novel Nrf2 activator. Inhibited airway inflammation to a similar degree as roflumilast in a mouse smoke exposure model. Improved urinary albumin/creatinine ratio in STZ-induced rat diabetic model (Type I) and KK-Ay mouse (Type II) and inhibited damage of the renal tissues. Synergistic effects by combination with losartan on urinary albumin/creatinine ratio were noted in KK-Ay mice. No adverse events were seen in multiple dose phase 1 study even at 1000 mg/man/day.
01/16/20	GEM141	Esophageal implant (See note)	Pediatric esophageal atresia and other conditions that affect the esophagus	Implant (autologous)	Cell therapy	Gastroenterology Regenerative medicine	IND ready	Esophageal implant made by combining a novel cell therapy platform (see TGEM38) with a patient's own cells (haematopoietic stem and precursor cells). GEM141 leverages the body's inherent capacity to heal itself as it is a "living tube" that facilitates regeneration of esophageal tissue and triggers a positive host response resulting in a tissue-engineered neo-conduit that restores continuity of the esophagus. These implants have the potential to dramatically improve the quality of life for children and adults
01/08/20	GEM140	Limbal stem cells	Intractable limbal stem cell deficiency	Implantation	Cell therapy	Ophthamology	Clinical	Establishment and production of limbus-derived epithelial cell plate manufacturing process in GMP facility with excellent economic feasibility. Phase 1 study is currently in progress.
01/08/20	GEM139	*Staphylococcus aureus* vaccine	*Staphylococcus aureus* infection	s.c.	Vaccine	Infection Vaccine	Preclinical	The vaccine comprising antigens and toxin is being developed. The candidate antigens to block the immune-evasion pathway by MSCRAMMs and toxin of S.aureus have already been defined.
12/24/19	GEM138	Biosimilar adalimumab	Same indications as adalimumab	i.v.	Antibody	Dermatosis Gastroenterology Immunology/ Inflammation Ophthamology Rheumatology	Preclinical	Purified GEM138 is highly similar to adalimumab by SDS-PAGE. GEM138 and adalimumab show a similar response in a TNF-a ELISA assay. Plant-based technology (TGEM036) was applied for production of GEM138.
12/24/19	GEM137	Biosimilar ranibizumab	Same indications as ranibizumab	Intra-vetreal	Antibody	Ophthamology	Preclinical	Peptide mapping by Mass Spectrometry confirms amino acid sequence identity of GEM137 with ranibizumab. Purified GEM137 is highly similar to ranibizumab by SDS-PAGE. Ligand (rVEGF) biding by GEM137 demonstrated to be similar to ranibizumab by ELISA. Plant-based technology (TGEM036) was applied for production of GEM137.
12/24/19	GEM136	Biosimilar trastuzumab	Same indications as trastuzumab	i.v.	Antibody	Oncology: Solid cancer	Preclinical	GEM136 N-terminal sequences are identical to trastuzumab. Levels of contaminating proteins, profiles of breakdown products and inhibitory activity to trastuzumab on in vitro growth of hER2 positive breast cancer cell line are similar between GEM136 and trastuzumab. Plant-based technology (TGEM036) was applied for production of GEM136.
12/20/19	GEM135	Inhibition of proteasome via novel target	Multiple tumors	i.v., i.p. and Oral	Small molecule	Oncology: Hematological cancer Oncology: Solid cancer	Preclinical	Novel target different from that for all the commercially available proteasome inhibitors. Works against many cancer cell lines tested including bortezomib, cisplatin and paclitaxel resistant cell lines. Significant therapeutic window between cancer and normal cells. Favorable toxicity profile. Regressed tumor growth and prolonged survival on syngeneic and xenograft mouse models. Expected to be effective against solid tumors without off target effects and peripheral neuropathy. Two lead compounds are being developed.
12/06/19	GEM134	Anti-CD147 antibody	Hematological (AML, MM etc) and solid tumors (liver, colon, lung etc)	i.v.	Antibody	Oncology: Hematological cancer Oncology: Solid cancer	Preclinical	Fully human antibody binding to human/ cynomolgus CD147. Has been shown to be very effective in various types of cancers in vivo xenograft mouse model. ADCC activity mainly contributes to the anti-tumor effect.
11/29/19	GEM058	Increase cellular ATP and promote wound healing	Diabetes foot ulcer	Topical	Small molecule	Dermatosis Metabolic disease	Phase 2 completed	Reducing inflammation of endothelial cells of blood vessels. Increasing cellular ATP and speeding up the healing of wounds by promoting the migration of epithelia cells in the skin of wounds. The arrangement of actin which is essential for cell migration is ATP dependent. Applicable to all kind of wound and low cost treatment

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11/25/19	GEM133	Myocardial protection by cardiac arrest temporally	Open heart surgery	Intra-coronary infusion	Others	Cardiology	Launched	GEM133 is a novel warm cardioplegic solution which in mixture with patient's oxygenated blood can produce effective and sustained cardiac arrest by a single dose 400ml. In addition, it has the following advantages; virtually unlimited aortic cross-clamp time, unassisted resumption of the cardiac rhythm, no ischemic and /or reperfusion injury, no need for cardiotonic support in the immediate postopertaive period.
11/15/19	GEM132	Matrix metalloproteinase-13 (MMP-13) inhibitor	Osteoarthritis (OA)	Intraarticular or Oral	Small molecule	Orthopedic	Preclinical	Extremely potent non-hydroxamic acid containing, non-zinc binding inhibitors of MMP-13 have been identified. High selectivity has been shown for this class of inhibitors over other MMPs. Lead inhibitor tested in the monoiodoacetate (MIA) rat model of OA and shown to protect cartilage when injected into the joint. Exhibits good oral bioavailability in the rat.
11/15/19	GEM131	Matrix metalloproteinase-2 (MMP-2) and MMP-9 inhibitor	Neuropathic pain and Amyotrophic Lateral Sclerosis (ALS)	Oral	Small molecule	CNS Pain/Neuropathy Rare disease	Close to IND ready *	Pain: GEM131 can block inflammatory responses at the site of nerve damage and has been shown to be efficacious in 4 different rodent models of neuropathic pain (spinal nerve ligation, chronic constriction injury of the infraorbital nerve, morphine withdrawal and thermal injury). ALS: Elevated levels of MMP-2 and-9 have been found in the skin and blood of people with ALS. Significantly improved larval locomotion in both the TDP-43 and SOD1 larvae models in Drosophila. Exhibits good oral bioavailability. *: Final stages of completion of IND enabling studies for both neuropathic pain & ALS
11/15/19	GEM130	Antiviral	Infections caused by herpes simplex virus in face and lip	Topical	Small molecule	Dermatosis Infection	Launched	• First cold sore product on the market that combines the therapeutic benefits of an antiviral with an innovative transparent bioadhesive film. • When applied to the lesion, generates a transparent film that acts as a bioadhesive sustained matrix release that improves product bioavailability while promotes itching reduction and wound healing. • Indicated for the topical treatment of symptoms (tingling, burning, discomfort) of recurrent herpes labialis caused by herpes simplex virus (VHS).
11/15/19	GEM129	Immuno-modulator	Anogenital warts, Actinic keratosis, Basal cell carcinoma	Topical	Small molecule	Dermatosis Infection Oncology: Solid cancer Urology	Phase 2	• The first product on the market that combines the therapeutic benefits of a marketed immunomodulator with an innovative transparent bioadhesive film. • When applied to the lesion, generates a transparent bioadhesive film, which acts as a reservoir or matrix release and reduces the local reactions and increases the permanence of the product in the action site. • The results of non-clinical studies demonstrate that GEM129 has a better safety profile with an equivalent efficacy than its reference product. Clinical studies on-going.
11/15/19	GEM128	Antibiotic	Primary and secondary skin infection - canine pyoderma	Topical	Small molecule	Others	Clinical for animal	• The first medicine on the market that combines the therapeutic benefits of a marketed antibiotic with an innovative film-forming, long-lasting delivery technology. • When applied to the lesion, generates a transparent film that acts as a bioadhesive sustained release matrix maintaining the optimum concentration of the antibiotic in the skin for a period of 6-8 hours. • The bioadhesive film generated reduces product removal from the area due to animal scratching, licking or friction with skin folds which contribute in improving the treatment efficacy. Also, reduces oral antibiotic overusing by improving topical treatment with this innovative technology. *Canine bacterial infections of the skin, including superficial pyoderma
11/15/19	GEM127	Antibiotic	Primary and secondary skin infection - Impetigo, folliculitis, furunculosis (human use)	Topical	Small molecule	Dermatosis Immunology/ Inflammation Infection	Launched	• The first product on the market that combines the therapeutic benefits of a marketed antibiotic with an innovative transparent film-forming and bioadhesive delivery technology. • When applied to the lesion, generates a film that acts as a bioadhesive sustained release matrix, maintaining the optimum concentration of antibiotic in the skin for a period of 6-8 hours. • The bioadhesive film generated prevents the removal of the antibiotic from the lesion and acts as a protective dressing that prevents infection spreading.

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11/5/19	GEM126	Selective estrogen receptor downregulator	ER+ advanced or metastatic breast cancer	Oral	Small molecule	Oncology: Solid cancer	Phase 1	• Both antagonizes and degrades ER alpha in cells to achieve the goal of blocking the estrogen signaling pathway. • Favorable oral pharmacokinetics in healthy rats and dogs whereas fulvestrant has a low bioavailability and can only be intramuscularly administrated. • Favorable preclinical in vitro and vivo single agent efficacy in inhibiting ER+ breast cancer cell proliferation, in models of tamoxifen-sensitive and tamoxifen-resistant breast cancer. • Highly selective, no effect on other kinases and receptors. • Can be licensed to global area with some linitation.
11/5/19	GEM125	Antiinflammatory and antifibrotic mono-unsaturated fatty acid (MUFA)	NAFLD, NASH	Oral	Small molecule	Immunology/ Inflammation Metabolic disease	Preclinical	MUFA (C16:1) is more capable of entering and improving function of endothelial cells and increasing cellular fluidity. Directly targets liver fat accumulation while also reducing LDL, increasing HDL and reducing Triglycerides. Targets F0-F2 Fibrosis, approximately 60%-70% of NASH patient population. More stable and less susceptible to oxidize/polymerize than Eicosapentanoic Acid derivatives. Can be used alongside drugs with alternate mechanisms of action or unwanted effects.
11/5/19	GEM124	Antiatherosclerotic and antiinflammatory mono-unsaturated fatty acid (MUFA)	Atherosclerosis (future indications: primary prevention of Heart Attack, Stroke and Death and Secondary Prevention of Myocardial Infarction in Europe)	Oral	Small molecule	Cardiology Immunology/ Inflammation Metabolic disease	Preclinical	MUFA (C16:1) is more capable of entering and improving function of endothelial cells and increasing cellular fluidity. Developed to target vulnerable, high risk plaques. Preclinical data shows superior plaque reduction over EPA. Directly targets fats while also reducing LDL, increasing HDL and reducing Triglycerides. More stable and less susceptible to oxidize/polymerize than Eicosapentanoic Acid derivatives.
10/29/19	GEM123	miRNA targeting cancer stem cells	Colon cancer	i.v.	Nucleic acid	Oncology: Solid cancer	Preclinical	GEM123 suppressed the stemness of cancer cells by inhibiting KLF5 expression, and provoked apoptosis and cycle arrest through the downregulation of TFDP1and MDM2 expressions. GEM123 also inhibited tumor growth with no apparent side effect in mouse model.
10/29/19	GEM122	Natural Killer T (NKT) cell-mediated anti-tumor responses	Solid cancer	i.v.	Cell therapy	Oncology: Solid cancer	Phase 1	GEM122 shows its anti-tumor activity by activating endogenous NKT cells. Activated NKT cells strongly enhance both innate and acquired immune systems, induce the long-term immune memory and promote accumulation of TILs (tumor infiltrating lymphocytes) in tumor sites. GEM122 consists of a novel NKT ligand and a novel APC. The novel NKT ligand shows much stronger NKT activity than the previous NKT ligand, α-Galactosylceramide and by using the novel APC, the cellular product can be manufactured efficiently as well.
10/23/19	GEM121	Modified phytic acid	Cancer	Oral	Small molecule	Oncology: Hematological cancer	Preclinical	• Demonstrated selective anti-cancer effects including apoptosis and inhibition of Akt activation. • In vivo study using mice with adult T-cell leukemia showed reduction of the size of the cancer.
10/23/19	GEM120	Inhibition of membrane binding of Pr55Gag	HIV	i.v.	Small molecule	Infection	Preclinical	• Inhibited the membrane localization of Pr55Gag and stopped budding of HIV virus. • Captures HIV in immune cells and induces apoptosis of the HIV-infected immune cells.
10/23/19	GEM119	Inactivation of end-product of lipid peroxidation	Cerebral Infarction	i.v.	Small molecule	CNS	Preclinical	• Showed more potent 4-hydroxynonenal - quenching activity compared with carnosine or histidine hydrazide (HH) at 30min incubation. • GEM119 (ip administration) rescued the hippocampal CA1 cell death of transient cerebral ischemia model of Mongolian gerbil whereas HH did not.

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10/23/19	GEM118	Suppression of TGF-β/Smad and related signaling	Systemic sclerosis	Oral	Small molecule	Dermatosis Rare disease	Preclinical	• Inhibited phosphorylation of Smad3 and expression of Col1a2, FN1 and CTGF stimulated by TGF-β in cultured human dermal fibroblasts. • Ameliorated bleomycin-induced skin fibrosis in both preventative and curative mouse model.
10/16/19	GEM117	Induction of apoptosis in adipocyte	Liposysis, Non-surgical fat reduction, Diabesity,	Sub-cutaneous	Small molecule	Metabolic disease	Phase 2a	• GEM117 specifically induce apoptosis on local injection site adipocyte • Phase 1 showed great safety without drug related SAE in 40 healthy volunteers • In pre-clinical study - Better efficacy on local fat reduction and less side effects that surpass the current surgical liposuction and other non-surgical products. - Has benefit effects on lipid and glucose metabolism and improves the indicator of diabetes
10/16/19	GEM116	Increase in lipolysis, fat and energy metabolism	Obesity, Diabesity, NAFLD/NASH	Oral	Small molecule	Metabolic disease	Phase 2	• GEM116 demostrated the saftest profile in its clinical phase 2 study with more than 200 patients without any drug related AE or SAE and potentially lower CVD risk seen by other competitors in the market. • Around 30% of subjects lost at least 5% body weight in 12 weeks, GEM116 shows around 5% body fat lost, lower TC by around 12mg/dL, and lower LDL by around 6 mg/dL •Pre-clinical study shows GEM116 has the potential to treat NAFLD/NASH via reducing liver fibrosis by aroud 40% .
10/16/19	GEM115	Trastuzumab biosimilar-ADC	HER2-positive metastatic breast cancer	i.v.	Antibody	Oncology: Solid cancer	Phase 1	Conjugate of trastuzumab biosimilar and DM-1. The profiles of trastuzumab of GEM115 and GEM115 are similar to Herceptin and Kadcyla, respectively, in peptide mapping, receptor binding affinity, inhibition of cell proliferation, ADCC activities, in vivo xenograft mouse model, PK etc. Potential Indication: Early Breast Cancer (adjuvant)
10/16/19	GEM114	Ophthalmic formulation of GEM113	Wet AMD	Intra-vetreal	Antibody	Ophthamology	Phase 1	GEM114 mainly distributed in retina, vitreous body and aqueous humor after intravetreal injection in animals. Potential Indication: Diabetic Macular Edema; Myopic Choroidal Neovascularization (mCNV); Retinal Vein Occlusion (RVO); Diabetic Retinopathy (DR)
10/16/19	GEM113	Bevacizumab biosimilar	Non-Squamous NSCLC	i.v.	Antibody	Oncology: Solid cancer	Phase 3	The profile of GEM113 is similar to Avastin, in peptide mapping, receptor binding affinity, inhibition of cell proliferation, in vivo xenograft mouse model, PK etc. Potential Indication: Metastatic Coloerectal Cancer; Recurrent Giloblastoma; Metastatic Renal Cell Carcinoma; Persistent Recurrent or Metastatic Cervical Cancer; Epithelial Ovarian, Fallopian Tube, or Primary Peritoneal Cancer
10/4/19	GEM112	Antibody against *H.pylori* -derived HSP60	Eradication of *H.pylori*	i.v.	Protein	Gastroenterology Infection	Preclinical	Antibiotic-resistance *H.pylori* are getting serious and antibiotic therapy failure rate is over 20%. GEM112 is a highly specific antibody against H.Pylori-derived HSP60 which inhibits T cell proliferation and induces IL-10 and TGF-b1. GEM112 does not bind to human HSP60.
10/4/19	GEM065	Fucosylation of CAR-T cell and TCR-T cells to improve homing to tumors, increased intra-tumor penetration and killing of cancer cells. Potential for enabling CAR-T cell therapies to achieve higher response rates for treatment of blood cancers and also work for treatment of solid tumors	Lymphoma, Leukemia, Melanoma, Lung and breast cancers are initial indications	Infusion	Protein	Oncology: Hematological cancer Oncology: Solid cancer	Preclinical; Human safety and efficacy observed in stem cell transplantation	Ex-vivo fucosylation kit using proprietary recombinant fucosyl-transferase enzymes aimed at improving efficacy, safety and cost of care for T-cell therapy not only for treatment of blood cancers but also importantly, enabling CAR-T cell therapy to work in the treatment of solid tumors . Similar technology as in GEM036 for hematopoietic stem cell therapy, but a different fucosyl-transferase is used. Fucosylation enhances homing and intra-tumor penetration of CAR-T, TCR-T, TIL/CTL cells for improved tumor killing. Fucosylation does not affect healthy tissues.

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9/30/19	GEM111	c-RAF allosteric inhibitor	Multiple cancers (lung, renal, liver etc)	Oral	Small molecule	Oncology: Solid cancer	IND	A second generation (novel salt) of the c-RAF allosteric inhibitor in GEM 110 allowing for a more patient-centric dose profile. The clinical safety profile of GEM110 thus far will be informative and additive to the current clinical program thereby significantly de-risk and accelerate the clinical stage development of GEM111.
9/30/19	GEM110	c-RAF allosteric inhibitor	Multiple cancers (lung, renal, liver etc)	Oral	Small molecule	Oncology: Solid cancer	Phase 1	The product is the combination of GEM110 and sorafenib. The product induces pDAPK and c-Raf to transfer from mitochondria to cytoplasm together with ROS increment resulting tumor cell death and demonstrated 73% and 57% tumor inhibition rate respectively in lung cancer and renal cancer animal xenograft model. FDA approved the clinical protocol to explore subjects by p-DAPK biomarker whose expressin could be related with tumor progression.
9/30/19	GEM108	Buccal Epithelial cells for re-epithelization	Urethral strictures	Implantation	Cell therapy	Regenerative medicine Urology	Phase 2b completed	Live cultured buccal epitherial cells indicated for urethral strictures. Excellent safety and efficacy profiles have been established through Phase IIb clinical trials. Potential label extension to rare disease in pediatric population; hypospadias.
9/30/19	GEM107	Chondrocytes for hyaline cartialge regeneration.	Articular cartilage defects	Interventional Implantation	Cell therapy	Orthopedic Regenerative medicine	Launch	Live cultured chondrocytes indicated for hyaline cartilage reneration in cartilage injuries. Over 600 pateints treated with several publications and patent protection.
9/30/19	GEM106	Osteoblasts for bone regeneration	Avascular necrosis/ Osteonecrosis	Interventional Implantation	Cell therapy	Orthopedic Rare disease Regenerative medicine	Launch	Live cultured osteoblasts indicated for bone repair and regeneration that stop progression of avascular necrosis. Over 400 pateints treated with GEM106. Potential label extension to different indications. This product has been granted Orphan Designation by the US FDA & EMA.
09/24/19	GEM105	Viscosupplementation	Knee osteoarthritis	Intraarticular	Poly-saccharide	Orthopedic	NDA application	Cross-linked sodium hyaluronate hydrogel with sodium hyaluronate fluid. Long lasting and sustainbale efficacy due to unique rheology of the cross-linked hydrogel. Injected only once and single injection shows comparable efficacy to a multiple-injection treatment. Phase-III clinical trial has been finished and NDA application has been submitted.
09/12/19	GEM104	Interleukin-1 beta inhibition	Various types of colitis (IBD, immune-related and chemotherapy-induced colitis)	Oral	Small molecule	Gastroenterology	Preclinical	-A known approved drug molecule developed for new therapeutic indications of treating and alleviating symptoms of various types of colitis. -Showed greater pharmacological effects than the reference drug, mesalazine, in reducing inflammatory colon swelling and intestinal ulceration, while restoring damaged intestinal membrane. -One of few products capable of repairing damaged intestine membrane caused by colitis. -Synergistic effect via different mechanism of actions, can provide greater medical benefits to patients suffering from IBD or various types of colitis caused by cancer treatment with chemo- or immuno-therapy.
09/03/19	GEM103	Iron chelator	Neurodegenerative and Ophthalmologic diseases including Traumatic brain injury (TBI) and AMD as lead indications. Additional indications in PD and ALS	Oral	Small molecule	CNS Ophthamology Rare disease	Phase 2 ready for TBI and Phase 1 ready for AMD	Novel oral iron chelator with excellent PK and ability to penetrate the brain and retina. Non-protein bound or "free" iron has been demonstrated to be deeply involved in neurodegenerative and ophthalmologic diseases, yet optimal chelators are not available. GEM103 efficiently removes free iron from the brain and retina. Biomarkers to guide drug development are available for both TBI and AMD. Phase 1 trials have been completed for transfusion-related iron-overload. Chronic toxicology completed.
08/21/19	GEM102	Cyclodextrin derivative	Cancer	i.v.	Small molecule	Oncology: Solid cancer	Preclinical	Modified Methyl-b-Cyclodextrin (CyD). Displayed potent antitumor activity in vitro, compared to M-β-CyD. Drastically inhibited tumor growth after a single intravenous injection to tumor-bearing mice, compared to doxorubicin and M-β-CyD, without any significant change in blood chemistry values.

Drug Candidates

GEMSEKI

As of 03/25/20

Date	Candidate	Mechanism of action	Indication	Route	Modality	Disease area	Development stage	Note
08/16/19	GEM101	Positive allosteric modulator of GABA-A receptor	Depression and PTSD	Intra-nasal	Peptide	CNS	Preclinical	Exhibited robust mixed anxiolytic and antidepressant activity in vivo animal models. Increased neurogenesis 1 week after single injection. No decrease in locomotor activity, no sedation.
08/16/19	GEM100	mGluR5 negative allosteric modulator	Depression and movement disorder	Intra-nasal	Peptide	CNS	Preclinical	mGLuR5 leader peptide was discovered and in vivo efficacy confirmed. GEM100 specifically increases locomotor activity in rats, with no effects on behavior.
08/16/19	GEM099	TrkB modulator	Depression	Intra-nasal	Peptide	CNS	Preclinical	TrkB peptide modulator discovery was launched.
08/13/19	GEM098	GnRH receptor antagonist	Endometriosis and Uterine fibroids	Oral	Small molecule	Endocrinology Gynecology	Phase 1	In Phase 1b, GEM098 showed dose-dependent suppression of LH, FSH and E2. The suppressive effects on E2 lasted up to 24 hrs and were more excellent when compared with the published phase 1 data of Elagolix in healthy premenopausal women. No serious adverse events were seen and well tolerated up to 320 mg QD.
08/13/19	GEM097	Factor VIIa derivative	Bypassing therapy in hemophilia with inhibitors	i.v.	Protein	Hematology	IND ready	GEM097 is rFVIIa fused to transferrin and has longer half-lives than rFVIIa in rats and monkeys. A cleavable linker between rFVIIa and trasferrin of GEM097 allows minimal reducuction of FVIIa activity due to fusion. Preclinical (GLP) toxicity studies did not show any toxic evidence in rats or monkeys.
07/31/19	GEM096	Progesterone receptor agonist	Recurrent preterm birth	Oral	Small molecule	Endocrinology Gynecology Rare disease	Phase 2 completed	Potentially the first oral hydroxyprogesterone caproate product candidate indicated for the prevention of recurrent preterm birth and has been granted orphan drug designation by the FDA. An end of Phase 2 meeting was completed with the FDA.
07/31/19	GEM095	Androgen receptor agonist	NASH, Cirrhosis	Oral	Small molecule	Metabolic disease	Phase 1	An oral prodrug of bioidentical testosterone that is being developed as a treatment of cirrhotic non-alcoholic steatohepatitis (NASH).
07/31/19	GEM094	Androgen receptor agonist	NASH, Pre-Cirrhosis	Oral	Small molecule	Metabolic disease	Phase 2 ongoing	An oral prodrug of bioidentical testosterone that is being developed as a treatment of non-alcoholic steatohepatitis (NASH) and is being studied in the LifT Phase 2 clinical study in biopsy confirmed NASH subjects.
07/31/19	GEM093	Androgen receptor agonist	Hypogonadism	Oral (QD)	Small molecule	Endocrinology Urology	Phase 2 completed	A novel next generation oral prodrug of testosterone with potential for once-daily oral dosing that has completed Phase 2 testing.
07/31/19	GEM092	Androgen receptor agonist	Hypogonadism	Oral (BID)	Small molecule	Endocrinology Urology	Phase 3 (a few months before PDUFA date)	A novel oral prodrug of testosterone that is designed to help restore normal testosterone levels in hypogonadal men. GEM092 was well tolerated and met the primary end-points in Phase 3 testing with twice daily dosing. Easy to use for patients and physicians to prescribe due to fixed doing regimen.
07/16/19	GEM091	Prevention of protein aggregation via increased intracellular ATP and increase of expression of tyrosine hydroxylase (TH)	Parkinson's disease	Oral Nasal	Small molecule	CNS	Preclinical	New treatment for Parkinson's disease. GEM091 increases intracellular ATP level and ATP is reported to boost protein solubility. GEM091 increases TH expression and dopamine production, reverses paraquat induced PD symptoms and improves behavior of 6-OHDA treated mice.
07/16/19	GEM090	Increase cellular ATP and promote wound healing	Epidermolysis bullosa (EB)	Topical	Small molecule	Dermatosis Rare disease	Preclinical	Increasing cellular ATP and speeding up the healing of wounds by promoting the migration of epithelia cells in the skin of wounds. Expected to shorten wound healing time and improve EB patients' QOL with a formulation optimized for EB treatment. Moreover, maybe reduces the risk of squamous cell carcinoma which is highly related to Dystrophic EB patients.
07/12/19	GEM089	Inhibition of pro-cytokines, enhancement of growth factor PDGF	Alzheimer's disease and Vascular dementia	Topical	Botanical	CNS	Phase 2	Small molecules from soybean extract. MOA is different from Tau and Amyloid mechanisms. Effective in $AlCl_3$ induced Alzheimer-like dementia model and bilateral common carotid artery occlusion induced vascular dementia model. In Phase 2 study, MMSE and ADAS-Cog for patients without any dementia medication indicated that 70-85% patients improved at weeks 4 and 12.

Drug Candidates

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Date	Candidate	Mechanism of action	Indication	Route	Modality	Disease area	Development stage	Note
07/12/19	GEM088	SSRI with agonist-antagonistic action on 5-HT receptors	Urinary incontinence	Oral	Small molecule	Urology	Phase 3 ready	Repositioning from another indication and has already a proven record of good safety in more than 300 patients with further 300 treated in phase 2. It has shown therapeutic activity in animal models of both stress and urge urinary incontinence. In phase 2 trials, clinically meaningful effects in mixed incontinence were shown as well as very good tolerability. The compound is ready to enter phase 3.
07/08/19	GEM087	Corticosteroid receptor agonist	Ocular inflammation and Meibomian gland dysfunction (MGD)	Topical (eye drop)	Small molecule	Ophthamology	Preclinical	New ophthalmic suspension of loteprednol etabonate (LE) with improved ocular bioavailability. - Drug exposure in aqueous humor and iris ciliary body are about 6 fold higher than Lotemax® in rabbits. - The new formulation of LE administered twice a day for the treatment of adjuvant induced chronic uveitis model in rabbits showed a similar efficacy with lotemax administered four times a day
07/08/19	GEM086	VEGF inhibitor	Wet AMD (age-related macular degeneration)	Topical (eye drop)	Small molecule	Ophthamology	Preclinical	New formulation of axitinib designed to deliver the drug across ocular tissues to choroid and retina. - Greatly enhancing aqueous solubility (4000-fold solubility enhancement) - High drug exposure in retina (300-fold of IC50) - Potential for substitution of anti-VEGF Ab treatment via intravitreal injection
07/08/19	GEM085	Cancers with moderate to high Her2 expression	Cancers with moderate to high Her2 expression	i.v.	Protein	Oncology: Solid cancer	Preclinical	The anti-Her2 ADCs consiting of trastuzumab and novel linker-drugs. - 10-100 fold higher potency comparting to Kadcyla® - High theraperutic window (MED ~1-2 mg/kg, MTD ~100 mg/kg in mice) - High tumor growth inhibition rate (90-100%) with high stability in vivo
07/04/19	GEM084	MRI contrast agent formulation	Diagnosis of bladder cancer	Topical (Intravesical)	Other	Diagnosis	Phase 2 ready	New formulation consisting of iron and gadolinium for intravesical instillation. A pilot trial in humans provided evidence that the MRI contrast agent can be detected in the bladder wall. No treatment-related AEs were observed.
07/04/19	GEM083	Vasoconstriction and anti-inflammatory action	Hemorrhagic cystitis	Topical (Intravesical)	Small molecule	Immunology/ Inflammation Oncology: Supportive care Rare disease Urology	Phase 2	Pre-liposomal lyophilate containing tacrolimus. POC achieved in animal models of both chemo-cystitis and radiation cystitis. POC achieved in first-in-man experience treating severe recurrent hemorrhagic cystitis. Orphan drug designation granted.
06/27/19	GEM082	Modified IL-1 receptor antagonist (IL-1RA)	Osteoarthritis, Gout, Anterior cruciate ligament (ACL) injury	Intra-articular	Protein	Immunology/ Inflammation Orthopedic	Preclinical	Single intra-articular injection of Anakinra (rhIL-1RA) improved OA symptoms vs placebo in clinical Phase-2 but the effect was short-lasting. GEM082 showed prolonged retention in the joint and sustained efficacy. GEM082 targets directly the biology of the inflammasome, involved in acute OA pain flares, without the adverse effects of steroids. Available for licensing to Japan, Korea, China and Taiwan.
06/12/19	GEM081	Oncolytic virus	Solid tumor (Melanoma, GI cancer etc.)	IM	Virus	Oncology: Solid cancer	Phase 2 ready	Genetically non-modified and non-pathogenic virus with - oncolytic, oncotropic and immunomodulating properties - excellent safety profile - various development options Medicine containing this virus is already approved for treatment for many years in certain countries. This virus has established fully GMP certified API production
06/06/19	GEM080	Novel cell surface receptor for insoluble protein involved in Parkinson's/Lewy body diseases	Parkinson's diseases, Lewy body disease	NA	NA	CNS	Preclinical	Cell surface receptors for insoluble protein involved in Parkinson's disease/Lewy body disease which were newly identified by utilizing artificial liposomes embedded with endogenous membrane proteins (see TGEM025 in Technology). Antagonists of the receptors are expected to be effective on Parkinson's/Lewy body diseases.

Drug Candidates

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Date	Candidate	Mechanism of action	Indication	Route	Modality	Disease area	Development stage	Note
06/06/19	GEM079	Novel cell surface receptor for angiogenesis regulation	Cancers, Chronic inflammation, Diabetic retinopathy, Myocardial infarction, etc	NA	NA	Cardiology Oncology: Solid cancer Ophthamology	Preclinical	Cell surface receptor involved in angiogenesis which was newly identified by utilizing artificial liposomes embedded with endogenous membrane proteins (see TGEM025 in Technology). Agonists of the receptor are expected to be effective on diseases where angiogenesis is involved as an aggravating factor.
05/30/19	GEM078	Hyaluronic acid-based nanocarriers of cisplatin	Head and neck cancer, Pancreatic cancer, Melanoma with metastasis	i.v.	Small molecule	Oncology: Solid cancer	Preclinical	CD44 targeting and higher stability lead to enhance lymphatic delivery and inhibit cancer with lymphatic metastasis. Chemodrug encapsulated by nanocarriers minimize systemic toxicities. Convenient dosing by intravenous injection.
05/30/19	GEM077	AMPK activator	Topical fat accumulation	Transdermal or oral	Small molecule	Metabolic disease	Preclinical	A small molecular AMPK activator. It inhibited the growth of adipocytes in vitro and suppressed body weight and fat increases in vivo. Topical used formulation is under testing.
05/30/19	GEM076	Galectin-12 inhibitor	Seborrheic dermatitis; Sebaceous hyperplasia	Transdermal	siRNA	Dermatosis	Preclinical	A modified siRNA for suppressing gene expression of galectin-12 which is a lipid droplet protein and regulates lipid accumulation and lipolysis. The siRNA can reduce the lipid in sebocytes and adipocytes and shows good stability and selectivity to reduce the lipid accumulation through transdermal delivery in vivo.
05/30/19	GEM075	Novel functional excipient	Oral formulation (e.g. direct compression, granulation, solid dispersion)	Oral	Polymer	Others	Preclinical	The synthetic polyvinyl acetate (PVAc)-based polymer of functional excipient is utilized as solubilizer that could increase drug solubility and enhance drug absorption. This novel excipient has better flowability, easy for use and widely application. This excipient will be useful for new drug and insoluble drug development.
05/30/19	GEM074	Dual inhibitor of Topo 1 and Topo 2	Drug resistant cancers	i.v.	Small molecule	Oncology: Solid cancer	Phase 1	1st-in-class combo therapy, covalently conjugated to simultaneously inhibit Topo 1 and 2, w/ EGFR cross-talk and extremely low toxicity for high dosing. Reduced tumor sizes w/ 38% CR and 98% TGI (compared to 0% and 33%, respectively, for imatinib) in CML xenograft models; and 98% TGI in colon cancer models at low doses. Showed immunotherapy enhancement effects with anti-PD-1 Ab and anti-CTLA-4 Ab with 100% CR in ovarian cancer model. No toxicity in rat & monkey models; No SAEs reported in Phase I at high dose 180mg/m2. Excellent PK profile and GI permeability of 9.2 enabling oral formulation.
05/30/19	GEM073	Kinase inhibitor of TGFβ-mediated phospho-SMAD2 signal transduction	COPD, IPF, Lung cancer	Oral	Small molecule	Immunology/ Inflammation Oncology: Solid cancer Respiratory	Phase 1	This kinase is selectively expressed in resident macrophages and airways epithelia of the lung and upregulated in COPD and IPF patients. A highly selective inhibitor showed efficacy across at least 3 different animal models relevant to COPD, IPF and NSCLC. Phase 1 trial has completed with a clean safety and tolerability profile.
05/30/19	GEM072	Orally available somatostatin analogues	Metabolic syndromes, Acromegaly, Hyperprolactinemia etc.*	Oral	Protein	Endocrinology Gynecology Metabolic disease	Preclinical	The technology and "know-how" to synthesize somatostatin analogues which will be more potent, more specific, stable and orally available have been established. A few analogues (at lead generation/optimization stage) with different combination of receptor selectivity and differential hormonal secretion inhibition properties are available. *: Congenital hyperinsulinism, insulinomas, glucagonomas
04/02/19	GEM071	Nanoparticl formulation of 2-deoxy-glucose	Hepatocellular carcinoma, Renal cancer, Colorectal canser	i.v.	Small molecule	Oncology: Solid cancer	Preclinical	In the xenograft model, the administration of nanoparticle formulation once a week showed superior antitumor effect than daily administration of 2-deoxyglucose alone. No side effects were observed. Enhanced the antitumor effect by combination with existing anticancer drugs. Enhanced T cell infiltration into tumor tissue. The substrate used in the nanoparticle formulation are used in approved medicines (FDA).

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Date	Candidate	Mechanism of action	Indication	Route	Modality	Disease area	Development stage	Note
04/02/19	GEM070	Nanoparticl formulation of ɤ-Oryzanol	Diabetes, Hyperlipidemia, Menopausal disorder, Irritable bowel syndrome, etc.	Oral	Small molecule	Endocrinology Gastroenterology Metabolic disease	Preclinical	Improved intestinal absorption and shows a drug effect in an extremely small amount (1/1000 of normal particles). Reduced preference for high fat diet. Suppressed ER stress enhancement on hypothalamic/pancreatic islet. Improved abnormal glucose metabolism and abnormal lipid metabolism. The substrate used in the nanoparticle formulation are used in approved medicines (FDA).
03/12/19	GEM024	1. Selective inducer of apoptosis through modulation of NF-kB/P53 axis. 2. Eliciting adaptive immune response by recruitment of T Cells to tumors.	Oral squamous cell carcinoma	Oral, pastille based topical delivery	Small molecule	Oncology: Solid cancer	Phase 2 ready	Updated on March 12, 2019 First-in-class, patented, combination therapeutic that simultaneously upregulates a cluster of genes promoting cell death and downregulates a cluster of genes promoting survival of cancer cells. Phase 1 results showed no significant AE, dose-dependent modulation of key biomarkers involved in disease pathogenesis, and T cell recruitment to tumor making it “hot”. FDA approved moving to Phase 2.
03/07/19	GEM069	Immuno-modulator (adjuvant)	Vaccine, Cancer immunotherapy etc.	Injection	Other	Immunology/ Inflammation Oncology: Solid cancer Vaccine	Preclinical	*E.coli* producing monophosphoryl Lipid A whose structure is similar to exsiting adjuvants such as MPL and GLA. Shows similar efficacy with MPL in vitro and in vivo. Lower cost production through simple fermetation and purification steps.
03/01/19	GEM068	Gene therapy for novel target	Lung and other cancers	i.v.	Nucleic acid	Oncology: Solid cancer	Preclinical	The expression of this gene is reduced in various cancers. Adenovirus expressing this gene inhibits the HIF-1α expression and proliferation of various cancer cells. Adenovirus expressing this gene suppresses growth of lung cancer in nude mice.
03/01/19	GEM067	c-Kit inhibitor	Diabetic macular edema & Retinopathy	Oral	Small molecule	Ophthamology	Phase 2a	Inhibits stem cell factor-induced hyperpermeability Reverses retinal vascular leakage in STZ-induced diabetic rats Improved compliance to administration (oral) than anti-VEGF therapy (intra-vitreal injection) Applicable to non-responders to anti-VEGF therapy Repositioning of a marketed drug
03/01/19	GEM066	Selective STAT3 inhibitor (DNA-based Decoy)	Head and neck squamous cell carcinoma and Non-small cell lung cancer (including exon 20 mutations)	i.v.	Nucleic acid	Oncology: Solid cancer	Pre-IND for systemic administration formulation	1st-in-class STAT3 decoy; Suppresses binding of STAT3 to genomic DNA; Inhibits proliferation and promotes apoptosis of many cancer cells; Suppresses expression of STAT3 target genes and tumor growth in animal models; Shows increased response in combination with cetuximab and also with PD-1; Human Phase 0 study (intratumor injection); Suppresses STAT3 target genes expression with one dose Does not affect normal oral keratinocytes; Exploratory animal toxicology studies show no significant adverse effects
03/01/19	GEM064	Selective inhibitor of Na_v channels	Pain	Oral	Small molecule	Pain/Neuropathy	Preclinical	Potent and selective inhibitors for Na_v 1.7/1.8 subtypes Effective in inflammatory & neuropathic pain states Exellent non-clinical ADMET profile No off-target activity, very good in vitro cardiac safety margin, non-mutagenic
02/12/19	GEM063	Novel 24hr ibuprofen patch	Local pain in sprains	Local	Small molecule	Orthopedic Pain/Neuropathy	Phase 1	High payload: Contains 200 mg ibuprofen, Constant delivery over 24 hours, Class-leading adhesion, Water-resistant, Comfortable to wear and remove Pre-clinical safety studies were completed 2017 with no concerns/issues Phase I PK and sensitisation/irritancy studies were successfully completed in 2018 with no concerns/issues

Drug Candidates

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Date	Candidate	Mechanism of action	Indication	Route	Modality	Disease area	Development stage	Note
01/11/19	GEM009	BET inhibitor	Cancer, RA	Oral	Small molecule	Oncology: Solid cancer Rheumatology	Preclinical	Updated on January 11, 2019 More potent enzyme inhibition and anti-tumor activities compared with competitors (more potent than GSK525762A and comparble to ABBV-075). Superior safety profile than competitors (no inhibition on hERG or CYP3A4) and can be applied to RA. Easier manufacturing due to absence of asymmetric carbon.
01/11/19	GEM003	Selective glucocorticoid receptor agonist	RA/OA, Please refer to Note	Local	Small molecule	Dermatosis Gastroenterology Immunology/ Inflammation Orthopedic Respiratory Rheumatology	Preclinical	Updated on January 11, 2019 Expected to reduce unfavorable responses of glucocorticoids by selectivity of action (transrepression>transactivation) Discontinued development for RA/OA. Available for repositioning. Possible indications: Atopic dermatitis, Psoriasis, Asthma, COPD, Inflammatory bowel disease etc.
01/11/19	GEM002	Kappa-opioid receptor agonist	Pain/Itching, Please refer to Note	Oral	Small molecule	Gastroenterology Pain/Neuropathy	Preclinical	Updated on January 11, 2019 Discontinued development for pain because of company strategy. Available for repositioning. Possible indications: Chronic pains (Back pain, Arthritis pain, Cancer pain, Post-herpetic neuralgia, Trigeminal neuralgia etc), Pruritus, Irritable bowel syndrome
01/11/19	GEM001	TRPV-1 agonist	Rheumatoid Arthritis, Please refer to Note	Oral	Small molecule	Dermatosis Gastroenterology Hematology Infection Metabolic disease Oncology: Hematological cancer Oncology: Solid cancer Pain/Neuropathy Rheumatology	Phase 2a	Updated on January 11, 2019 Potently inhibits TNF-a production by oral administration. Discontinued development for RA. Available for repositioning. Possible indications: Neuropathic pain, Crohn's disease, Systemic lupus erythematosus, Cachexia, Acute infectious disease, Allergy, Pyrexia, Anemia, Diabetes, Diseases related TNF-α (Colitis, Psoriasis ets.)
01/07/19	GEM062	Cannabinoid-releasing topical formulation (siustained release for about 24 hours)	Chronic pain, Sclerosis, Lupus, others	Topical	Small molecule	Dermatosis Pain/Neuropathy	Preclinical	Cannabinoid's utility: nausea and vomiting during chemotherapy, chronic pain, muscle spasms, epilepsy, sclerosis, lupus, schizophrenia etc. This sustained-release topical formulation has significant potential to help treat these disorders. Preclinical efficacy demonstrated in a cutaneous lupus rodent model using AEA.
01/07/19	GEM061	Curcumin-releasing topical formulation (sustained release for about 24 hours)	Please refer to Note	Topical	Small molecule	Cardiology Hematology Immunology/ Inflammation Orthopedic	Preclinical	Indication: Osteoarthritis, CV disease, chronic inflammatory disease, vascular disease (Sickle Cell) Note: Curcumin's utility: chronic pain, chronic inflammatory conditions such as osteoarthritis, vascular disease such as Sickle Cell and diabetes. This formulation breaks through the limitations of the poor bioavailability of curcumin with oral administration. Preclinical efficacy demonstrated in an rodent arthritis model and a rodent diabetes model.

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Date	Candidate	Mechanism of action	Indication	Route	Modality	Disease area	Development stage	Note
01/07/19	GEM060	Nitric Oxide-releasing topical formulation (sustained release for over 48 hours)	Please refer to Note	Topical	Small molecule	Dermatosis Otolaryngology	Preclinical	Indication: Acne, Atopic Dermatitis, Fungal diseases, Wound healing, Chronic rhinosinusitis, Diabetic foot ulcers, Raynaud's Phenomenon, Middle-ear infections, Erectile dysfunction, Others Note: NO function: Regulation of the vasculature (vasodilatory), broad spectrum antimicrobial activity, anti-inflammatory, anti-oxidant, wound healing, skin cell maturation and survival etc. Human POC already shown with NO in onychomycosis, genital warts, moscullum contagiosum, pulmonary hypertension, acne, atopic dermatits (preliminary); animal POC demonstrated in over 20 peer-reviewed papers. This formulation breaks through the limitations of sustained NO (topical) delivery.
12/27/18	GEM059	Recombinant Human Interleukin-1 Receptor Antagonist	Please refer to Note	IM	Protein	Gastroenterology Immunology/ Inflammation Infection Oncology: Supportive care Orthopedic	Phase 1	Indication: 1: Infection, as manifested by febrile neutropenia, in patients with malignancies receiving chemotherapy drugs. 2: Diarrhea in patients with malignancies receiving chemotherapy drugs. 3. Gout arthritis Note: The world's first multiorgan protection agent for tumor chemotherapy. Inhibits cell cycle progression of normal cells and renders them resistanace to chemotherapy. No effects on tumor growth and their sensitivity to chemotherapy Phase 1 : No dose limiting toxicity, None of subjects presented grade 3 or above chemotherapy-induced neutropenia or diarrhea. Gout arthritis: Well tolerated and safe for patients who are restricted from NSAID, glucocorticoid, or colchicine.
12/27/18	GEM057	Increase hair follicle ATP and delay senescence of dermal papilla cells	Alopecia	Topical	Small molecule	Dermatosis	Phase 2	Boosts the ATP of human follicle dermal papilla cells, thereby slowing down the aging speed and prolonging hair cycle. No side effects and shorter time to observe efficacy Human trial (Androgenetic Alopecia): significantly improved 37.5% vs 0% (placebo) during 2 months Phase 2 (Female pattern hair loss) : ongoing (comparison with minoxidil)
12/13/18	GEM054	Intracellular superoxide removing agent	Chemotherapy induced Peripheral Neuropathy (CIPN)	i.v.	Small molecule	Oncology: Supportive care Pain/Neuropathy	Phase 2	There is currently no approved drug to prevent or treat CIPN. Completion of Phase 2b study. Global Phase 3 started in US, Currently in preparation for Phase 3 in Japan, South Korea, Taiwan and Hong Kong. Can be licensed to South Korea, Taiwan and Hong Kong.
12/13/18	GEM053	Substance for covering oral lesions (no active ingredients: medical device)	Control and relief pain of oral mucositis by chemotherapy/radiotherapy	Local	Small molecule	Oncology: Supportive care Pain/Neuropathy	Launch	There is no standard treatment for chemo/radiotherapy induced mucositis–high unmet medical needs. Forms a mucoadhesive barrier film when applied in the oral cavity. Excellent control of oral pain (rapid and long-lasting effects and ready to use)

Drug Candidates

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Date	Candidate	Mechanism of action	Indication	Route	Modality	Disease area	Development stage	Note
12/13/18	GEM052	Mitochondria-targeted apotosis inducer	Relapsed/Refractory Peripheral T-cell Lymphoma (PTCL) and other hematological cancers	i.v.	Small molecule	Oncology: Hematological cancer	Phase 2	No approved drugs for PTCL indication in Europe (3 drugs have been approved in Japan and US). Less toxic compared with competitors approved in Japan and US. US Phase 2a: 5 responders in 11 refractory PTCL and DLBCL patients, and 2 responders in 7 PTCL patients. Applicable to relapsed and refractory PTCL. Phase 2 Pan-Asia pivotal study is ongoing. Can be licensed to US/EU and China (except Beijing, Shanghai, and Guangzhou).
12/10/18	GEM051	Protective agents from heat stress	Heatstroke	Oral	Other	Immunology/ Inflammation	Preclinical	Suppression of vascular endothelial cell damage and production and release of inflammatory cytokines from blood cells due to heat stress. Ingredients derived from citrus fruit extract.
11/22/18	GEM050	Curcumin analogue	CML, Pancreatic cancer, Glioblastoma etc.	Oral	Small molecule	Oncology: Hematological cancer Oncology: Solid cancer	Preclinical	Inhibited proliferation of CML and pancreatic cancer cells at the submicromolar level. Unlike imatinib, the inhibitory action is irreversible. Suppressed almost completely human CML cell growth without significant changes in body weight and peripheral white blood cell count in vivo. An incease in ROS/RCS produced by inhibition of their scavenging enzymes is assumed to be involved in anti-tumor action. Induced M phase arrest.
11/15/18	GEM049	Pan-NOX inhibitor	IBD, IPF, Neurodegenerative diseases	Oral	Small molecule	Gastroenterology Immunology/ Inflammation Pain/Neuropathy Respiratory	Preclinical	Highly potent NOX inhibitor : 20~50 times more potent than GKT-137831 Significant effects in DNBS-ulcerative colitis and LPS-induced acute inflammatory animal studies. Also showed positive results in IPF animal model. High oral bioavailability and clean off-targets profile.
10/25/18	GEM047	Lactoferrin complexes	Pain on Peripheral Neuropathy, Cancer	i.v.	Protein	Oncology: Solid cancer Pain/Neuropathy	Preclinical	Improved PK profile over natural lactoferrin. Analgesic effect on periferal neuropathy induced by oxaliplatin. Inhibitory effects on the growth of syngeneic murine lung cancer model Inhibitory effects on the growth of human non-small cell lung cancer cell in vitro. Positive effect of treatment on sepsis mice model.
10/25/18	GEM046	Indirect activator of AMPK	Hyperlipidemia, type 2 diabetes, Cancer	Oral	Small molecule	Metabolic disease Oncology: Hematological cancer	Preclinical	Derivative of fungus product ascochlorin. Good PK profile in rats. Superior effects than metformin on blood glucose and triglyceride level in diabetic mice model. Significant enhancement of the efficacy in combination with metformin on T2D model Significant enhancement of the antitumor activity of anti-PD-1 antibody in mouse model
09/20/18	GEM045	Autotaxin Inhibitor	NASH, Panacreatic cancer	Oral	Small molecule	Immunology/ Inflammation Metabolic disease	Preclinical	Showed antifibrotic efficacy with significant histopathological score reductions in chronic pancreatitis and NASH (Stelic STAM & MCD) in mice. Showed significant anti-inflammatory effects in paw edema animal study.
09/20/18	GEM042	TLR4 antagonist	NAFLD, NASH, AIH, CLD and CD	Oral	Small molecule	Gastroenterology Immunology/ Inflammation Infection Metabolic disease Rare disease	Phase 2	NAFLD (nonalcoholic fatty liver disease): The phase 2 results demonstrated significant improvement on relevant diagnosis and biomarkers. NASH: has recently been approved by US FDA for Phase 2 trial (Feb in 2020) AIH (autoimmune hepatitis; orphan designation): The phase 2 (open label) results will be available soon. CLD(chronic liver disease by HCV infection): A strong trend of improvement of liver function and safety in Phase 2. CD (Crohn's disease): Good efficacy in three Phase 2 POC studies. The drug is safe and tolerable in these trials. Can be licensed to territories except Asia

Drug Candidates

As of 03/25/20

Date	Candidate	Mechanism of action	Indication	Route	Modality	Disease area	Development stage	Note
08/31/18	GEM040	Topical anti-inflammatory	Joint pain, Muscle pain, Gout, Local inflammatory pain	Topical	Small molecule	Orthopedic Pain/Neuropathy	Preclinical	Topical formulations of Ibuprofen, Naproxen, Diclofenac to use in the treatment of inflamatory pain and related conditions Formulation shows 5 to 10X increase human skin permeation coupled Potential for OTC or RX introduction: minimal development timeline
08/31/18	GEM039	Antifungal	Onychomycosis	Topical	Small molecule	Dermatosis Infection	Preclinical	Novel and unique topical formulation of Terbenafine with exceptional permeation (40 fold) across the human nail. Potential for OTC or RX introduction: minimal development timeline
08/31/18	GEM038	Locally acting anti-inflammatory- Trigeminal neuroinflammation	Migraine	Local	Small molecule	Pain/Neuropathy	Phase 2a	Clinical POC in acute migraine therapy in Phase 2a Efficacy comparable to Triptans but with no systemic side effects or restrictions Shortened development timelines (NDA:2021) Product opportunity for use in Temporomandibular Joint Disease and trigeminal neuralgias
08/31/18	GEM037	Allosteric modulator of the CCR3 receptor	Asthma, Rhinitis	Oral	Small molecule	Immunology/ Inflammation Otolaryngology Respiratory	Phase 2a	In phase 2a: Highly significant effects on the methacholine provocative response Showed trends to improvement in EAR (Early Phase Allergic Response) Reduced induced sputum eosinophil percentage and increased percent blood eosinophil
08/27/18	GEM036	Hematopoietic stem cell fucosylation	Prevention of infection & GvHD from hematopoietic stem cell transplantation	Infusion	Protein	Immunology/ Inflammation Oncology: Hematological cancer Regenerative medicine	Phase 3 ready with FDA SPA	In Phase 2 study: Statistically significant acceleration of immune system reconstitution (neutrophil/platelet recovery) Significantly reduced infection and GvHD Improved survival Positioned to be best-in-class No reports of adverse event specifically attributable to fucosylation
08/07/18	GEM034	Derivative of neuroprotective protein	Stroke, Huntington chorea, Schizophrenia and PTSD	i.v.	Peptide	CNS	Preclinical	A cell-permeable recombinant peptide. Can cross the blood-brain barrier, is resistant to degradation, and can bind constitutively to its substrates. Significantly reduces brain damage in rodent stroke model. Expected to be treated after stroke without diagnosis of stroke type before dosing.
08/07/18	GEM033	Inhibitors of bacterial resistance mechanisms	Gram-negative MDR bacterial infections, Lung infections in cystic fibrosis (CF) patients	Intravenous, Aerosol, Topical	Small molecule	Infection	Preclinical	Restore effectiveness of shelved antibiotics. Lower the effective dose of antibiotics. Mitigate antibiotic resistance. Disrupt biofilm-based infections. Over 1,000 compounds with lead compounds for each indication identified.
07/26/18	GEM032	Calcium release-activated calcium channel inhibitor	Respiratory & Immuno-inflammatory diseases	Oral	Small molecule	Immunology/ Inflammation Respiratory	Phase 1	Preclinical study demonstrated the therapeutic potential in respiratory diseases causally associated with allergic inflamation. Phase 1 SAD/MAD study were completed.
07/26/18	GEM031	Store-Operated Calcium Entry (SOCE) Inhibitor	Lymphomas & Solid cancer	Oral	Small molecule	Oncology: Hematological cancer Oncology: Solid cancer	Phase 1	Demonstrated preclinical activity in a broad range of cancers. Phase 1 dose-escalation study in patients with relapsed or refractory lymphomas is ongoing.
07/26/18	GEM030	PI3K δ/γ dual inhibitor	Hematological malignancies, T cell Lymphoma, Hodgkin Lymphoma	Oral	Small molecule	Oncology: Hematological cancer	Phase 2	Highly specific dual PI3K δ/γ inhibitor with nano‑molar inhibitory potency Single-agent and combination with immune checkpoint inhibitor programs Inhibits primary patient leukemic/lymphoma cells Dose escalation study demonstrated an acceptable safety and tolerability with promising clinical activity

Drug Candidates

As of 03/25/20

Date	Candidate	Mechanism of action	Indication	Route	Modality	Disease area	Development stage	Note
07/20/18	GEM029	A cancer stem cell-associated transcription factor inhibitor	Malignant gliomas including glioblastoma (GBM)	Oral	Small molecule	Oncology: Solid cancer	Phase 1 ready	Directly kills the cancer stem cells. Significantly improves survival in orthotopic GBM PDX models. Inhibits tumor growth in a mouse implanted with GBM cells and the growth inhibition is augmented by combination with temozolomide/radiation. No significant toxicities are identified at therapeutic doses.
07/09/18	GEM028	miRNA targeting refractory colon cancer with mutated K-ras and refractory pancreatic cancer	Pancreatic cancer Colon cancer	i.v.	Nucleic acid	Oncology: Solid cancer	Preclinical	The miRNA regulates K-ras, Bcl2, survivin, and NF-kB and demonstrate excellent antitumor effect in vitro and in vivo.
07/09/18	GEM027	miRNA targeting refractory colon cancer with mutated K-ras	Colon cancer	i.v.	Nucleic acid	Oncology: Solid cancer	Preclinical	The miRNA regulates EGFR signaling pathway by directly inhibiting of both KRAS and AKT1 and demonstrate excellent antitumor effect in vitro and in vivo.
07/09/18	GEM026	siRNA suppressing the expression of novel cancer stem cell gene "Gene A"	Cancer	i.v.	Nucleic acid	Oncology: Solid cancer	Preclinical	Novel cancer stem cell gene "Gene A", which was discovered by single cell analysis of cancer stem cell, shows character as follows. 1.Superior cancer stem cell diagnostic marker than known cancer stem cell marker CD44v9 2.SiRNA targeting Gene A demonstrate excellent antitumor effect with currnet medicines.
07/09/18	GEM025	miRNA suppressing the expression of a protein characteristic of pancreatic cancer stem cells	Pancreatic cancer	i.v.	Nucleic acid	Oncology: Solid cancer	Preclinical	Excellent antitumor effect was demonstrated in uniquely established pancreatic cancer stem cell model in vitro and in vivo.
07/05/18	GEM021	Opioid and non-opioid analgesics with respiratory stimulant	Pain	Oral	Small molecule	Pain/Neuropathy	Preclinical (close to IND)	Combination of generic opioid/non-opioid analgesics with a generic respiratory stimulant using a regulatory approach known as the 505(b)2 submission. First drug introduction therapeutically equivalent to Vicodin® that prevents overdose death, deters abuse and prevents addiction. Low cost of goods.
06/04/18	GEM020	3rd generation immunotherapy targeting little gastrin	Gastro-intestinal cancer (Pancreatic)	s.c.	Protein	Oncology: Solid cancer	Preclinical	Applied 3rd generation immunotherapy technology to little gastrin. In-vivo POC has been validated in non-human primates.
06/04/18	GEM019	3rd generation immunotherapy targeting HER2	HER2 overexpressing cancer (Breast)	s.c.	Protein	Oncology: Solid cancer	Preclinical	Applied 3rd generation immunotherapy technology to HER2. Superior efficacy to trastuzumab and pertuzumab. In-vivo PoC has been validated in non-human primates.
06/04/18	GEM018	3rd generation immunotherapy technology	Cancer	s.c.	Protein	Oncology: Hematological cancer Oncology: Solid cancer	Discovery	Unique technology that triggers B and T cells simultaneously while activating all possible natural tumor killing mechanisms available to the immune-system. Tumor-directed specific activation reduces side effects to the minimum. Applicable on a target basis.
04/10/18	GEM008	Selective cMET inhibitor	NSCLC and HCC etc.	Oral	Small molecule	Oncology: Solid cancer	Phase 2	Effective on exon 14 skipping xenograft Development by an originator has been discontinued.

Technologies

As of 03/25/20

Date	Number	Technology	Summary
03/06/20	TGEM047	Innovative transparent film-forming and bioadhesive delivery technology	TGEM047 is a versatile topical bioadhesive film-forming vehicle (platform) with occlusive or semi occlusive characteristics and sustained release properties for an adequate vehiculization of lipophilic and hydrophilic components. It can also be used as a vehicle for the inclusion of other technologies (micro, liposome, nanoparticles etc). This technology has been successfully applied to different topical molecules demonstrating an improvement on the API's bioavailability profile as well as providing more adequate formulations for patient treatment adherence.
03/06/20	TGEM046	Proliferation control of Mononegavirales using photoswitching system	Mononegavirales are promising tools as oncolytic vectors and transgene delivery vectors for gene therapy and regenerative medicine. By using the specifically designed proteins, which reversibly heterodimerize upon blue light illumination, photocontrollable mononegaviruses (measles and rabies viruses) were generated. The proteins were inserted into the flexible domain of the viral polymerase, and the oncolytic virus showed strong replication and oncolytic activities only when the viral polymerases were activated by blue light illumination. Treatment of this oncolytic virus resulted in a substantial reduction in tumor growth and prolonged survival under the blue light.
03/06/20	TGEM045	Superior gene expression using photoswitching system	Fusion system of CRISPR-dCpf1 and novel photoswitching system. An improved split dCpf1 activator, which has the potential to activate endogenous genes more efficiently than a previously established dCas9 activator. The split dCpf1 activator can efficiently activate target genes in mice and provides an efficient and sophisticated genome manipulation in the fields of basic research and biotechnological applications.
03/06/20	TGEM044	ON/OFF control of mice genome recombination using photoswitching system	Fusion system of Cre–loxP recombination system and novel photoswitching system. Enables sharp induction (up to 320-fold) of DNA recombination and is efficiently activated even by low-intensity illumination (~0.04 W m-2) or short periods of pulsed illumination (~30s). Allows for efficient DNA recombination in an internal organ of living mice through noninvasive external illumination using an LED light source.
03/06/20	TGEM043	ON/OFF control of gene expression using photoswitching system	Fusion system of CRISPR-dCas9 and novel photoswitching system. Enables high blue-light-inducible activation of endogenous target genes in various human cell lines. Induced neuronal differentiation in iPS cells by achieving the activation of target genes.
03/06/20	TGEM042	ON/OFF control of gene editing using photoswitching system	Fusion system of CRISPR-Cas9 and novel photoswitching system. Induces targeted genome sequence modifications through both nonhomologous end joining and homology-directed repair pathways in response to blue light irradiation and can be switched off simply by extinguishing the light. Determines time-specific and location-specific activation by the irradiation.
03/06/20	TGEM041	Novel photoswitching system for optogenetic control of gene editing and expression	This system consisting of two proteins can control the activity of cellular proteins by the optogenetic method. These new proteins were engineered to enhance light-induced heterodimerization and show faster kinetics than any of the other conventional dimerization-based blue spectrum photoswitches. This is a powerful tool that can optogenetically manipulate molecular processes in biological systems.
02/18/20	TGEM040	Genome editing technology using oligonucleotides	The genome editing technology uses oligonucleotides only and requires neither protein nor double-strand break. Editing is highly selective and there are no by-product, which enables editing as intended without off-target risks. Easy administration to living body.

Technologies

As of 03/25/20

Date	Number	Technology	Summary
02/12/20	TGEM039	Multi Targeted Drug Delivery using Peptide Drug Conjugates (PDC's)	A novel Smart, Multi-Armed linker which is patented. The linker can bind up to three payloads –chemotherapeutic agents for treatment or fluorescent for diagnostics. The linker releases the payloads only in the Tumor cells and only after release the drugs become active and the fluorescent changes colors or starts to shine. We proved that Conjugates with dual drug payloads (multi-loading) resulted in enhanced cytotoxic effect towards cancer cells and less drug resistance evolved in comparison with mono-loaded counterparts. The novel linker can be used for PDC's, Antibody DC's(ADC's) and Nano-particle DC's(NDC's). An innovative technology for PDC's - a unique technology to synthesize cyclic peptides which are stable, selective and non-immunogenic. We can identify Receptors which are overexpressed in certain forms of cancer and synthesize peptides which will bind to these receptors so that we can use them for Targeted Drug Delivery.
01/16/20	TGEM038	A novel cell therapy platform to regenerate tubular organs	Proprietary and biocompatible scaffold (temporary cell delivery device) is combined with a patient's own cells (haematopoietic stem and precursor cells) to create an esophageal implant that could potentially be used to treat pediatric esophageal atresia and other conditions that affect the esophagus. TGEM038 can also be extended to other tubular organs, including the bronchi and trachea.
01/08/20	TGEM037	Novel *Escherichia coli-Mycobacterium* shuttle vector	TGEM037 has the following strengths compared to other vectors and expected to induce enhanced immune responses. • High stability and compatibility • High copy number in mycobacteria • Stable expression of exogenous genes in mycobacteria
12/24/19	TGEM036	*Staphylococcus aureus* vaccine	*Staphylococcus aureus* infection
11/29/19	TGEM035	Effective platform using highly branched glucan for DDS carrier	Highly branched glucan has very attractive characteristics, such as a spherical nano-sized particle, high water solubility, narrow molecular size distribution, and the numerous modifiable residues, as a dendrimer for pharmaceutical application. Established technology enables to control the average particle size of this glucan between 10 nm and 50 nm strictly, and to conjugate the functional substances, such as peptide, nucleotide, sugar chain, anticancer drug, and antibody, on the surface of its particle arbitrarily. The structural features of this polymer could permit drug delivery to specific tissues and multivalent interactions with target molecules. This glucan is an effective platform for DDS carrier such as vaccine and anticancer drug.
11/05/19	TGEM034	Innovative platform technology for targeted delivery of therapeutics	A proprietary biomimetic vector (a short peptide that binds to proteoglycan-rich tissues) can be fused with a wide variety of therapeutics (proteins, small molecules etc) and enables delivery of locally injected therapies to the hard-to-reach tissues where they are needed. Sustained therapeutic levels within the targeted tissues can increase efficacy and avoid systemic adverse events.
11/05/19	TGEM033	Long-term sustained release microformulations	This technology platform enables long-term sustained release of a drug into the blood from the administration site and treatment with once in 1 month to 6 months. The best polymer among more than 15 kinds of polymers can be proposed based on the results of feasibility studies. Opportunity for using this technology is limited to Japanese companies.
09/30/19	TGEM032	Cell Therapy technology platform for developing cell-based products for multiple chronic diseases	Novel and propreitary cell therapy platform technology that can be used for the development of cellular therapies. Cellular therapies are the new modality of treatment that provides long-term cures for chronic diseases. This platform can be applied across multiple disease areas-musculoskeletal disorders,urogynacology, oncology,cardiology. Three pronged approach to tackle degenerative diseases through drug/biologic/device mechanisms.
08/21/19	TGEM031	Delivery of anticancer drugs to cancer cells	TGEM031 is a modified β-Cyclodextrin and increased in vitro antitumor activities of doxorubicin (DOX), vinblastine and paclitaxel. The complex of DOX with TGEM031 markedly increased antitumor activity of DOX, after intravenous administration to tumor-bearing mice.
07/31/19	TGEM030	Innovative technology enabling improved GI absorption of the insoluble drug	A patented technology based on lipidic compositions which form optimal dispersed phase in the gastrointestinal environment for improved absorption of the insoluble drug. TGEM030 enables development of superior oral products with: Improved solubilization and high drug loading capacity, improved bioavailability, faster and more consistent absorption leading to reduced variability and reduced sensitivity to food effects. TGEM030 utilizes bioacceptable excipients and conventional manufacturing processes.

Technologies

As of 03/25/20

Date	Number	Technology	Summary
07/08/19	TGEM029	Sustained release PLGA	Poly lactide-co-glycolide acid (PLGA) microsphere technology for sustained release of drugs. Versatile drug release profiles could be achieved by adjusting the formulation compositions and effective plasma drug concentration could be maintained for several weeks or months upon one injection. In addition, the pharmacokinetic and pharmacodynamics evaluation models for sustained release technology have been established which could speed up product development.
07/08/19	TGEM028	Posterior eye delivery	An ocular delivery technology specially designed to topically deliver hydrophobic small molecular across ocular tissues into posterior ocular tissues. The eye drop based delivery technology can overcome the delivery obstacle of tissue barriers to transport therapeutics to posterior ocular target tissue. This breakthrough technology is expected to bring broader applications for posterior ocular drug delivery
07/08/19	TGEM027	Site-specific linker toxin	The disadvantages of traditional conjugation technologies include the lack of specificity at the connecting positions of the antibodies and the variable number of connections. The new technology can overcome these disadvantages by improving homogeneity of ADC via site-specific conjugation. The site-specific linker-toxin shows better homogeneity, stability and efficacy.
07/08/19	TGEM026	Intracellular delivery	This technology enables peptide and oligonucleotide drugs to be delivered efficiently into cells through conjugating a cell-penetrating motif (CPM) onto drug candidate. Unlike liposomal or other nanoparticle formulation, the CPM technology requires no encapsulation process and provides formulated drug product with high stability and storage condition tolerances.
06/06/19	TGEM025	Innovative proteoliposome methodology for rapid discovery of biomarkers, ligands and/or receptors	A newly developed one-step direct transfer technology for MALDI-mass spectrometry (MS) can eliminate time-consuming intermediate processes and separate or remove plasma high abundant proteins, and therefore is useful for rapid and efficientt peptide profiling of biological samples. In addition, this technology can be used for rapid identification of ligands and cell surface receptors including GPCRs and GPI anchors by combining with a library of membrane proteins keeping binding capability reconsitututed on artificial phopholipid bilayers of liposomes.
05/30/19	TGEM024	Novel lymphatic delivery system	A novel hyaluronic acid-based nanocarriers that could deliver more drug to lymph nodes. This delivery system may offer significant advantages for the use of platinum medicines in the management of locally advanced cancers. Organic solvent-free nanocarriers process. Active targeting to lymph node and tumor.
05/30/19	TGEM023	Innovative formulation for insoluble drugs	Novel platform of formulation design and evaluation include concept of formulation design, composition of formulation, in vitro dissolution study, and in vivo absorption test. This new concept of formulation design utilizes solubility buster with traditional excipient to resolve solubility problem of drugs. This platform could be widely applied to BCS II drugs and shorten development process. Traditional excipient and solubility buster are commercial products that are easy to purchase without limitation.
04/02/19	TGEM022	Innovative nanoparticle formulation	Achieved higher content of drug, more homogeneous particle size distribution, lower cost (1/10) and easier mass production compared to conventional methods. Easy to control particle size (2 nm ~ 500 nm). Provide DDS function to the drug and stay the drug in the cell for a long time. Enable re-development of compounds that abandon development with side effects and insufficient effect. The substrate used in the nanoparticle formulation are used in approved medicines (FDA).
02/12/19	TGEM021	A unique and effective transdermal delivery technology for small molecule drugs	Can formulate patches with very high payload: Up to 50wt% achieved for drug and excipients, Extended release (24h up to 7-day) formulations possible, Fantastic adhesion, Water-resistant , Comfortable to wear and to remove. Efficient release of drug with improved skin penetration through formulation of free base/acid of API (not a salt)

Technologies

As of 03/25/20

Date	Number	Technology	Summary
01/11/19	TGEM020	Corporate alliance program with prestigious academia	One of the best universities in the world is looking for corporate partners for its corporate alliance program. The program typically takes 3 years, and the corporate partner is expected to fund projects jointly undertaken by the university and the partner. The partner will have options to exercise further developments from the result of the projects. The university carefully selects its partners.
01/07/19	TGEM019	Novel patented topical delivery system	The delivery particles are extremely effective at lodging into the stratum corneum, where they persist and gradually release their payload over a 24-48-hour time period. The payload can be most any small molecule or peptide. Eventually, the particles slough off as part of the skin-regeneration process. The particles can be easily tuned to control release rate. Depending on the chosen payload and the clinical objective, the particles can be as small as ~200 nanometers in size...and up to ~5 microns. Demonstrated efficacy and safety in ~25 animal models of disease. Breakthrough technology with strong IP protection.
12/20/18	TGEM018	Internalizing antibody discovery (Only for partnership)	1) Screen hybridoma clones secreting cancer specific internalization antibodies with a patented high throughput assay 2) Panning living cancer cells with in-house constructed human naive ScFV library, wash away cell surface binders, apply cell lysate as sub-library; After several rounds of panning, enrich phages displaying antibodies with internalization ability
12/20/18	TGEM017	Novel bispecific antibody discovery (Only for partership)	IgG like bispecific antibody with new designed Fc mutations on top of "knob into hole" to enable super high heterodimer formation and purification as easy as a monoclonal antibody. Two versions have been developed: 1) Version 1: Two heavy chains plus one common light chain -Common light chain can be designed from two parental light chains by modeling and structure analysis if they share more than 80% homology -Biophysical and biochemical features of the two arms can be designed to meet functional needs (for example, imbalanced binding for CD20 and CD3 to balance safety and efficacy) and developability needs (to enable better isolation of heterodimer from homodimers) 2) Version 2: Two heavy chains plus two light chains -VH1/VL1 interface and VH2/VL2 interface on the 3D model will be analyzed first to select those less likely allowing VL exchange and then optimized for reduced frequency of VL exchange -The two arms will be differentiated for better heterodimer purification (similar to that in 1)
12/20/18	TGEM016	Antibody Humanization And Optimization	Detailed sequence analysis and 3D structure modeling Identification of the best human germline sequence templates, grafting the rodent or rabbit antibody CDR regions into the human antibody frameworks Identifying back-mutations and removing “hot spots” with developability issues High throughput lead selection and evaluation Efficient lead preparation for research purpose in house (>45 projects have been successfully completed!)
12/20/18	TGEM015	Therapeutic Antibody Discovery	Antigen design via bioinformatics and computer aided design Antibody development via hybridoma and/or phage display technology Screening mAb clones for high affinity, high expression, good efficacy and good developability
12/10/18	TGEM014	Determination method for NK cell activity	Simple and quick method for determination of NK cell activity which is applicable to whole blood. The amount of IFN-γ or TNF-α produced by activated NK cells in whole blood is used as an indicator of NK cell activity. Neither radioactive isotopes nor separation steps from blood cells are required.

Technologies

As of 03/25/20

Date	Number	Technology	Summary
10/09/18	TGEM013	SUMO-fusion protein expression technology	Enables the efficient lower-cost production and purification of high quality, correctly folded proteins useful in all applications of protein preparation and drug discovery. High expression levels Variations optimized for insect, mammalian, E.coli and yeast. Non-exclusive licensing.
10/09/18	TGEM012	The methods and tools relating to UPS	The methods and tools for discovery of ubiquitin pathway system (UPS) enzyme functions and modulating molecules. This technology enables the following assays. HTS and validation assays of deubiquitinase (DUB) activity and E2, and E3 ligase Cellular ubiquitylation activity We are launching a new proteomics service that utilizes the TUBE technology to get better mass spec data about ubiquitylated proteins.
08/31/18	TGEM011	Novel topical drug delivery technology	Enables superior transdermal penetration of active ingredients to the target tissue Suitable for most APIs and drug classes Minimizes systemic exposure and side effects Improved API photostability (No degradation upon sun exposure) Formulated with all GRAS (generally recognized as safe) excipients
08/27/18	TGEM010	Fucosylation technology for cell immunotherapy	Improved delivery of therapeutic cells Applicable to hematopoietic stem cells, cytotoxic and regulatory T cells (Treg), CAR-T, mesenchymal stem cell and NK cells Simple and rapid procedure
07/09/18	TGEM009	Technology establishing cancer stem cell	Established the cancer stem cell model that can produce in vivo tumor from one cancer stem cell. In vivo tumor were formed from 10 cells in cell lines established from patient derived tissue of metastatic colorectal cancer as well as from in vitro cell lines.
07/05/18	TGEM008	A platform technology that allows any cell to continuously produce a bioluminescent signal representative of its real-time metabolic activity level	This technology removes steps from, and allows the continuous measurement of, cell-based in vitro and animal imaging-based in vivo assays. No negative effects on host cells and correlation with alternative assays are validated. Licenses available for pre-made cell lines or the technology platform.