



**MEDPACTO**

*Genome-based innovative drug discovery company*

# Investor Event

November 15<sup>th</sup>, 2023


# Forward-looking Statement of MedPacto

This presentation contains statements regarding **MedPacto's (the "Company's")** future financial performance, business development strategy and plans, anticipated clinical trials, results and regulatory approvals that constitute forward-looking statements as meant by the United States Private Securities Litigation Reform Act of 1995. No forward-looking statements from the Company can be guaranteed. All statements that are not historically stated facts are, or may be construed as forward-looking statements. Actual results may differ materially from those expressed in, or implied by these statements due to factors including, but not limited to:

- (i) the Company's ability to achieve expected clinical, regulatory and contractual milestones on expected timelines or at all,
- (ii) difficulties or delays in the development and commercialization of new products,
- (iii) difficulties or delays in clinical trials, manufacturing, distribution, sale and licensing of our products as performed by the Company or by the Company's representatives,
- (iv) new regulations and laws,
- (v) the Company's ability to obtain, protect and enforce patents and other intellectual property,
- (vi) financial and political instability including overall economic conditions,
- (vii) unfavorable outcomes in proceedings of a legal or regulatory nature,
- (viii) risks associated with portfolio actions including acquisitions, divestitures, collaborations and joint ventures.

These and other important factors are discussed in Korean in the Company's annual and quarterly reports available from the Financial Supervisory Service's website ([dart.fss.or.kr](http://dart.fss.or.kr)) or from MedPacto's website or from the Company's Investor Relations. Forward-looking statements and clinical data in this document are presented only as of the specified date on this document. Unless obliged by applicable regulations, the Company is under no obligation to publicly update any of the provided information. This presentation contains financial information prepared under Korean IFRS (K-IFRS) but may contain certain non-generally accepted accounting principles ("GAAP") financial measures to describe the Company's performance. Investors should review our publicly filed reports in their entirety and not to rely on any single financial measure.

# Agenda

- 
- 01** Key Takeaways
  - 02** Pipeline & Programs
  - 03** Vactosertib
  - 04** MP2021
  - 05** Clinical Advisory Board



# MEDPACTO

An oncology-focused clinical stage biotech company



➤ Phase 2b/3 clinical trial is set to begin with mOS 17.35, ORR 18.75% (300mg BID) in Non-MSI-H Colorectal Cancer, a globally leading best-in-class data

➤ Founder/CEO, a distinguished scholar of Tumor Micro-environment leads R&D efforts.

➤ Meeting global New Drug Application (NDA) standards is made possible through the wealth of clinical development expertise established through collaborative partnerships with top-tier pharmaceutical companies such as AZ, MSD

➤ Expanding our first-in-class novel drug pipelines and accelerating the clinical development while actively pursuing potential licensing opportunities

## Late-stage and early-stage therapeutics based on innovative targets developed in-house

### ✓ Colorectal Cancer (Vactosertib + Keytruda)

- **Demonstrated Best-in-class data in MSS-type Colorectal Cancer** at the ESMO2023 data presentation

Overview of Efficacy (RECIST)	300mg BID (N=32)	Overall (N=105)
ORR	13.33%	18.75%
mOS	17.35	15.8

Prolonged survival by almost 7 months in comparison to current standard treatment regimens, including Regorafenib (mOS 6.4), Lonsurf (mOS 7.1), and the recently approved Trifluridine/Tipiracil + bevacizumab (mOS 10.8).

- **With the goal of becoming a market leader in the MSS CRC**, the Phase 2b/3 global clinical trial is set to begin in 2024.













### ✓ Osteosarcoma (Vactosertib Monotherapy)

- **For Osteosarcoma, a disease with no cure, received several designations** including FDA Orphan Disease Designation (ODD), Pediatric Rare Disease Designation (RPDD), Rapid Approval Designation (FTD), and European Orphan Drug Designation (OMPD).
- A full-scale clinical trial in the United States has been ongoing since early May 2023. The goal is to secure a program for swift market entry.

### ✓ Pre-clinical stage programs

- **MP 2021; KDDF-selected program**, is set to receive government support. Having completed the patent application, conducting presentations and data release at various conferences and academic societies. Currently gathering GLP-Tox data and the submission of the IND will commence in 2024.
- Other First-in-class pipelines; **Actively pursuing out-licensing opportunities.**

Transforming the landscape of global oncology with robust portfolio and first-in-class pipeline

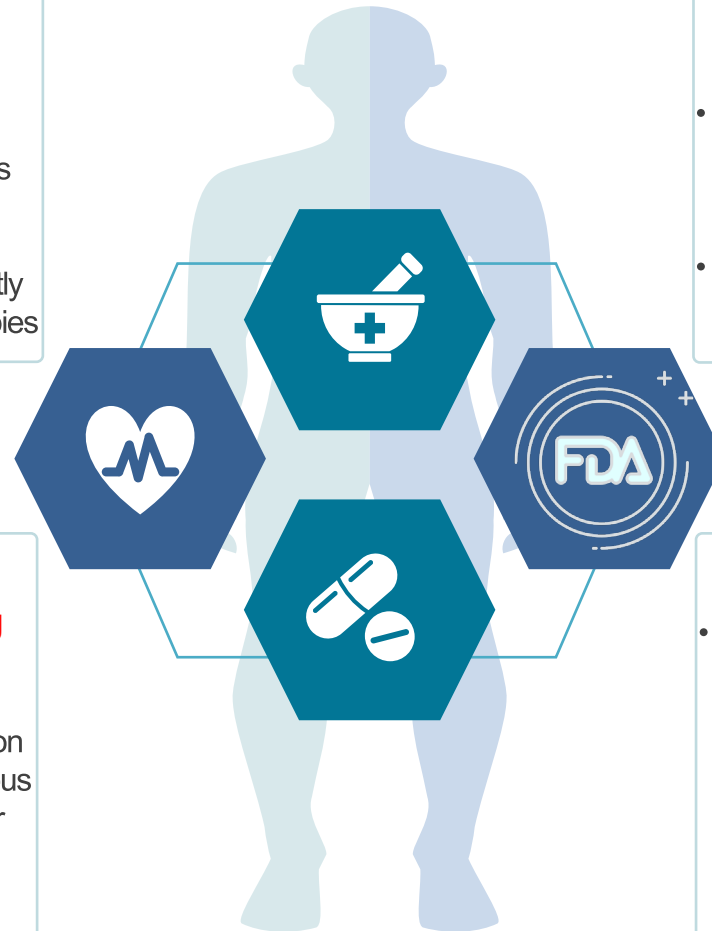
Product	Type	Indication	Pre-clinical	IND-enabling Studies	Phase1	Phase2	Phase3	Collaborators	
Clinical-stage Programs	Small Molecule	Colorectal Cancer	Vactosertib + Keytruda (anti-PD-1)						
		Colorectal Cancer	Vactosertib + Keytruda (anti-PD-1)						
		Osteo-sarcoma	Vactosertib Monotherapy						
		NSCLC 1L	Vactosertib + Keytruda (anti-PD-1)						
		NSCLC 2L	Vactosertib + Imfinzi (anti-PD-L1)						AstraZeneca 
		Bladder Cancer	Vactosertib + Imfinzi (anti-PD-L1)						AstraZeneca 
Pre-clinical Programs	Biologics	Rheumatoid Arthritis	MP2021						
		TBD	MP2021						
	Cell Therapy	TBD	IND-Enabling						

### 3. Vactosertib\_Key Factors

Best-in-class small molecule TGF- $\beta$ 1 receptor kinase inhibitor,  
showcasing exceptional data compared to the Standard of Care in Colorectal Cancer

#### Optimizing the TME for Treatment

- By suppressing the overexpressed TGF- $\beta$ 1 signaling within the tumor microenvironment, Vactosertib disrupts the formation of extracellular matrix barriers around cancerous tissues
- Playing a pivotal role in transforming 'cold' tumors into 'hot' tumors resulting in significantly enhance the effectiveness of a cancer therapies



#### Potential market leader in Colorectal Cancer

- The combination therapy of Vactosertib and Keytruda has demonstrated superior performance compared to global rivals and the standard of care in MSS Colorectal Cancer
- Phase 2b/3 trial IND application will commence in 2H 2023.

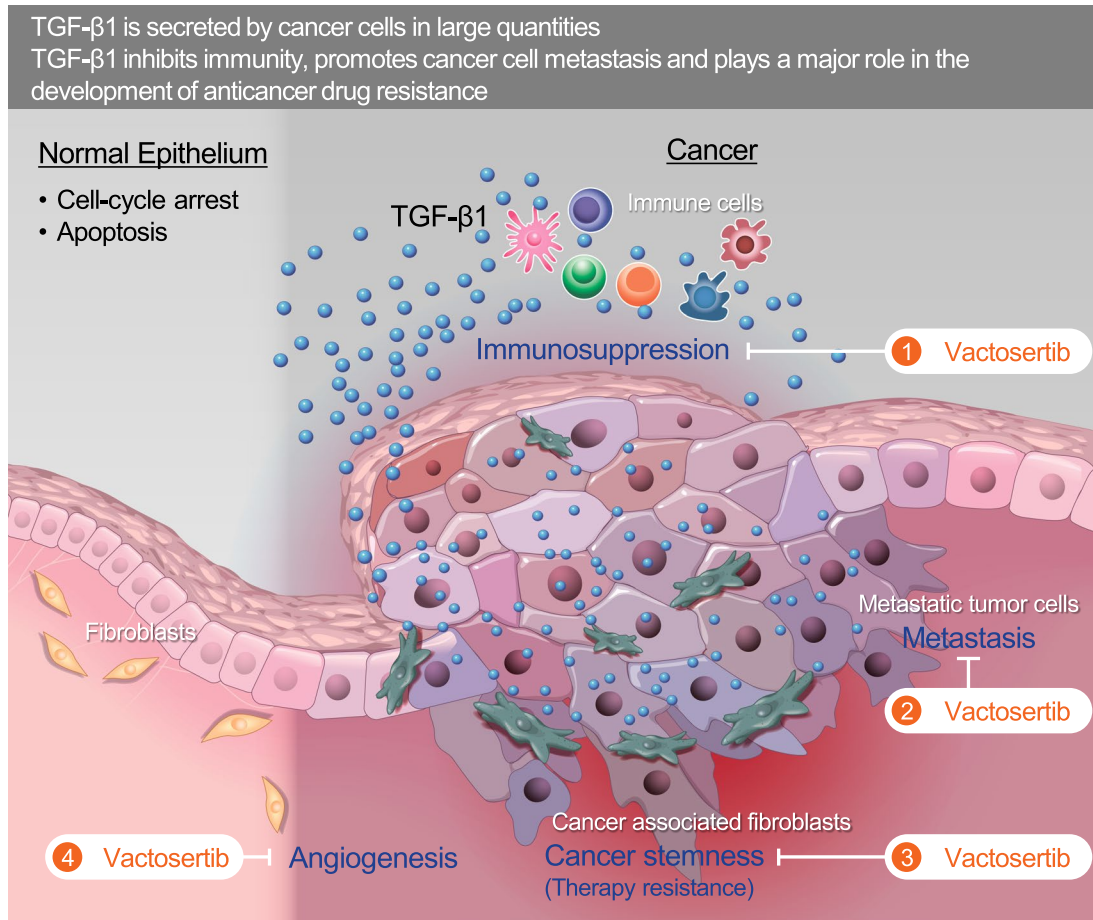
#### Market potential as a blockbuster drug candidate

- The evident synergistic potential in combination with diverse anticancer therapies across various cancer types strengthens its attractiveness for potential out-licensing.

#### U.S. FDA & EMA approved programs

- Vactosertib, for the treatment of Osteosarcoma, has received approvals from the U.S. FDA and EMA, securing designations such as "Orphan Drug," "Fast-Track," "Rare Pediatric Disease" by the FDA, and "EMPD" by the EMA emphasizing its potential to address critical medical needs, expedite development, and underscore innovation in pediatric oncology.

## TGF-β1 regulates the tumor microenvironment and promotes tumor growth and metastasis



### Main Functions of Vactosertib

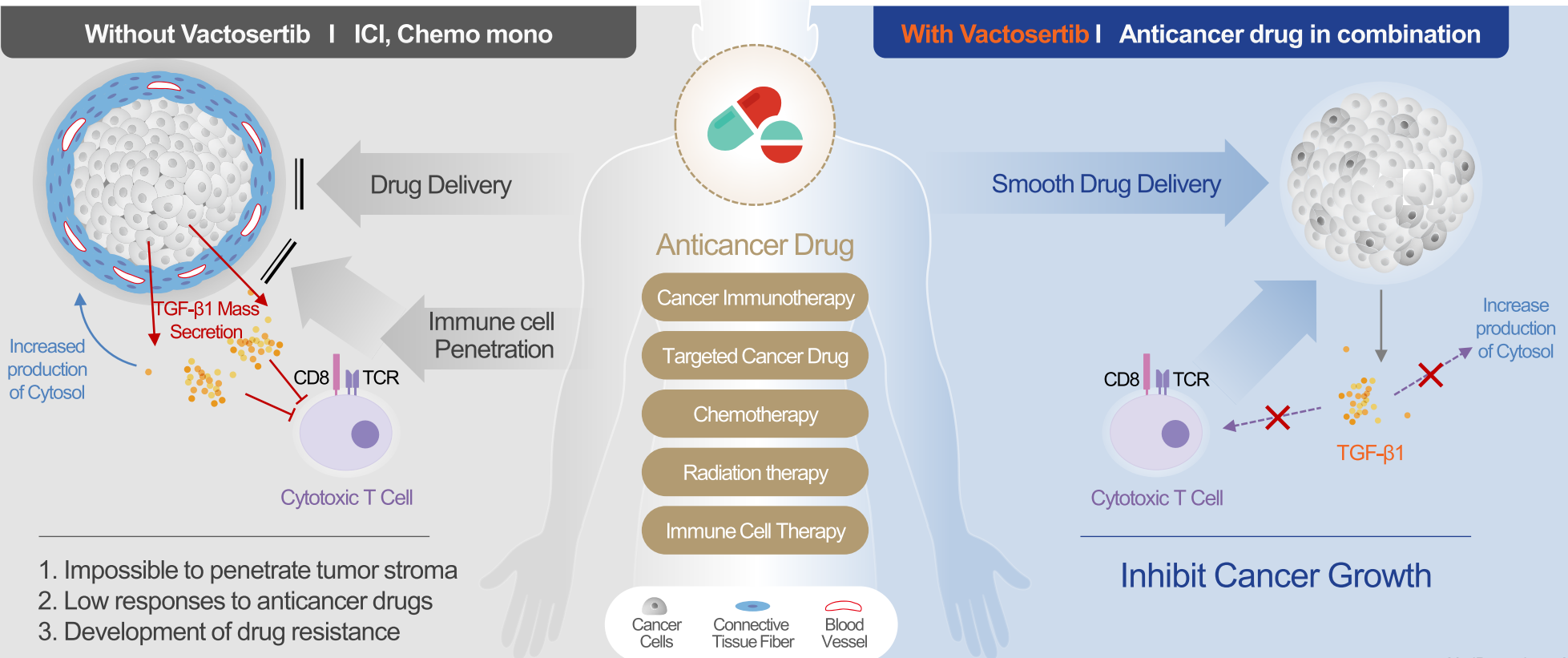
- 1 Promotes tumor cell killing activity by immune cells
  - Activates cytotoxic function of T- and NK cells
  - Inhibits activity of regulatory T-cells
  - Prevents T-cell exhaustion
- 2 Inhibits Metastasis
  - Inhibits epithelial-mesenchymal transition (EMT), cell migration and metastasis
- 3 Inhibits cancer stem cell production
  - Suppresses TGF-β1-induced generation of cancer stem cells (cause of drug resistance to various anti-cancer drugs such as Gleevec, a treatment for CML, and paclitaxel)
- 4 Inhibits angiogenesis



### 3. Vactosertib\_MOA of Vactosertib

#### Vactosertib can be combined with many different existing cancer treatments

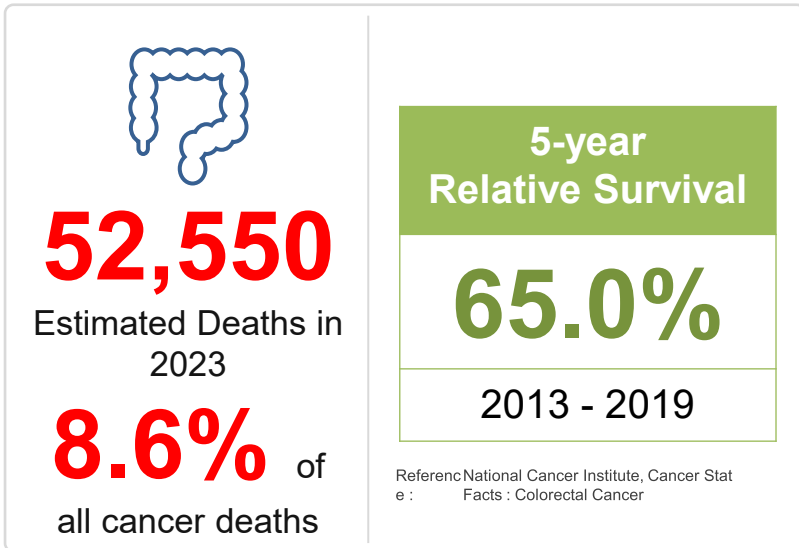
- TGF-β1 acts on stromal cells around cancerous tissues to produce large quantities of extracellular matrix, creating a barrier surrounding the tumor  
→ prevent anticancer drugs and immune cells from attacking cancer tissue
- Vactosertib, a TGF-β1 signaling inhibitor, prevents the formation of matrix walls around cancerous tissues  
→ Various cancer therapies help attack cancer cells



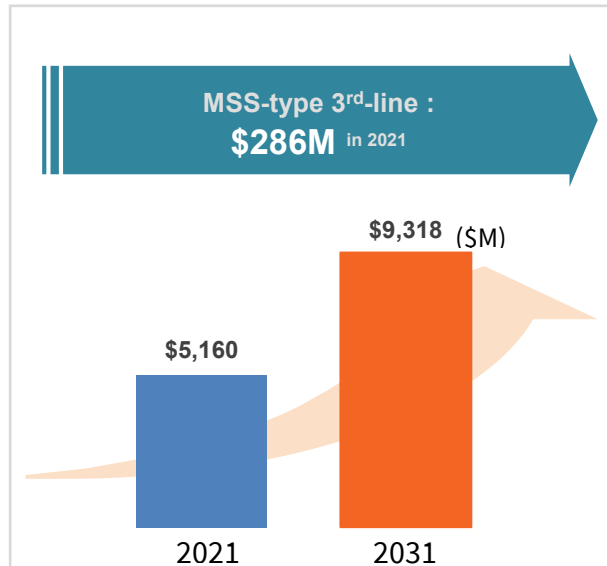
1. Impossible to penetrate tumor stroma
2. Low responses to anticancer drugs
3. Development of drug resistance

The demand for innovative and effective colorectal cancer treatments is increasing, driven by a significant patient population and a rising incidence rate

**Colorectal cancer stat Facts**



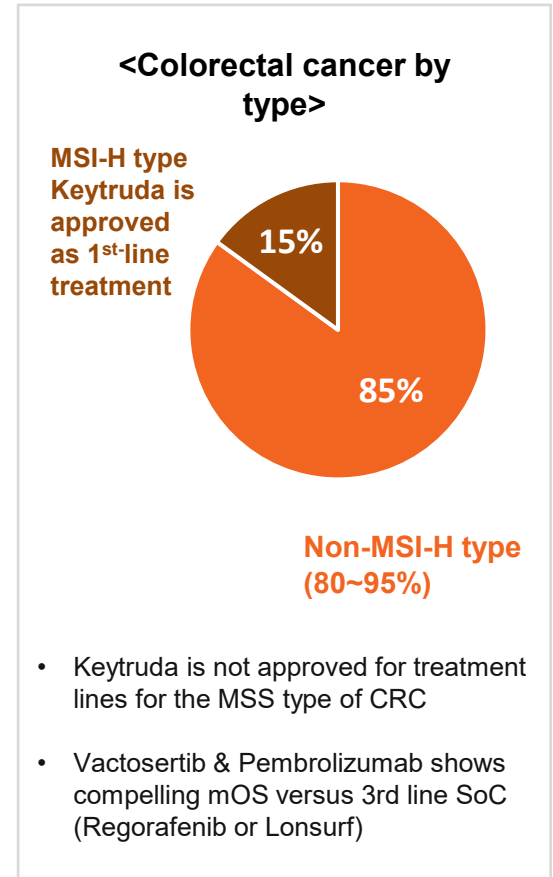
**Rising Colorectal Cancer Market**



- 85% of addressable CRC market does not show response to just I/O
- Opportunity to dominate market as first I/O combo to reach the market

Ref. Data monitor

**MSS CRC Target Indication**



**Growing need for new therapies**

Colorectal cancer stands as a primary contributor to cancer-related morbidity and mortality on a global scale. This underscores the **pressing need for groundbreaking therapies** that have the potential to enhance **patient outcomes** and **elevate the quality of life**.

### 3. Vactosertib\_ Combination with pembrolizumab – Leading the class in terms of efficacy data

Promising clinical outcomes demonstrate a outperforming effectiveness in reaching areas of metastatic colorectal cancer that single agents cannot access

#### Interim Efficacy Results from the MSS-CRC Phase 1b/2a Clinical Trial

##### Comparing with Standard of Care

Outcomes	Phase2 Vactosertib for CRC (Vactosertib+Keytruda)		Regorafenib mono	Lonsurf mono	Avastin + Lonsurf
mOS	<b>15.80 months</b> (Overall)	<b>17.35 months</b> (300 BID)	6.4 months	7.1 months	10.8 months
ORR	<b>13.33% (14/105)</b> (Overall)	<b>18.75% (6/32)</b> (300 BID)	1% (5/505)	1.6% (9/534)	TBD (490)

##### Clinical trials ongoing/completed in MSS-CRC

Atezolizumab + cobimetinib	Pembrolizumab mono	Regorafenib + Avelumab	Pembrolizumab + Lenvatinib	Regorafenib + Nivolumab
8.9 months	5.0 months	10.8 months	7.5 months	11.9 months
2.7% (5/183)	0.0% (0/18)	0% (0/43)	22% (7/32)	7.1% (5/70)

- Planning US FDA IND submission for **Phase 2b/3 (pivotal trial)**
- Target Patients: 3<sup>rd</sup>-line treatment for Recurrent, Refractory or Progressive Non-MSI-H colorectal cancer
- Multicenter (US, KR), Randomized design

Reference : Vactosertib Study MP-VAC-204

GI ASCO: Trifluridine/tipiracil plus bevacizumab for third-line treatment of refractory metastatic colorectal cancer: The phase 3 randomized SUNLIGHT study.

Regorafenib(Stivarga) mono : Highlights of prescribing information, Revised 09/2012

Lonsurf mono : Mayer RJ, Van Cutsem E, Falcone A, et al. Randomized Trial of TAS-102 for Refractory Metastatic Colorectal Cancer. NEJM. 2015;372:1909-1919.

Atezolizumab+cobimetinib : The Lancet Oncology June 2019, Pages 849-861

Pembrolizumab : le et al. New Eng J MED(2015)

Lonsurf + Nivolumab : 10.1200/JCO.2019.37.8\_suppl.48 Journal of Clinical Oncology 37, no. 8\_suppl (March 10, 2019) 48-48

Regorafenib+Avelumab : Cancer Treat Rev. 2018 Jan;62:61-73. doi: 10.1016/j.ctrv.2017.10.011. Epub 2017 Nov 10.

Pembrolizumab+Lenvatinib : 2021 ASCO Gastrointestinal Cancers Symposium

Clinical outcomes demonstrate a favorable safety profile in metastatic colorectal cancer

### Summary of Treatment Emergent Adverse Events

Summary of Treatment Emergent Adverse Events	Overall, (N=105) n(%), [E]	200mg QD, (N=30) n(%), [E]	200mg BID, (N=36) n(%), [E]	200mg TID, (N=7) n(%), [E]	300mg BID, (N=32) n(%), [E]
<b>TEAE</b>	90 (85.71), [691]	23 (76.67), [97]	31 (86.11), [272]	7 (100.00), [57]	29(90.63), [265]
<b>TEAESI</b>	2 (1.90), [2]	2 (6.67), [2]			
<b>Immune-related TEAE</b>	26 (24.76), [95]	8 (26.67), [16]	6 (16.67), [22]	1 (14.29), [1]	11 (34.38), [56]
<b>Grade 3-5 TEAE</b>	33 (31.43), [74]	10 (33.33), [13]	7 (19.44), [29]	4 (57.14), [9]	12 (37.50), [23]
<b>TEAE related to Dermatology</b>	51 (48.57), [125]	13 (43.33), [28]	17 (47.22), [28]	3 (42.86), [11]	18 (56.25), [58]
<b>TEAE related to Adrenal Insufficiency</b>	2 (1.90), [2]		1 (2.78), [1]		1 (3.13), [1]
<b>Serious TEAE</b>	20 (19.05), [27]	6 (20.00), [6]	6 (16.67), [10]	1 (14.29), [1]	7 (21.88), [10]
<b>Serious TEAE related to Vactosertib</b>	9 (8.57), [11]	1 (3.33), [1]	2 (5.56), [2]		6 (18.75), [8]
<b>Discontinue due to TEAE</b>	9 (8.57), [10]	3 (10.00), [3]	3 (8.33), [3]		3 (9.38), [4]

<sup>†</sup>Abbreviations: TEAE, treatment-emergent adverse event; n, No of subjects with adverse event; E, No of adverse event

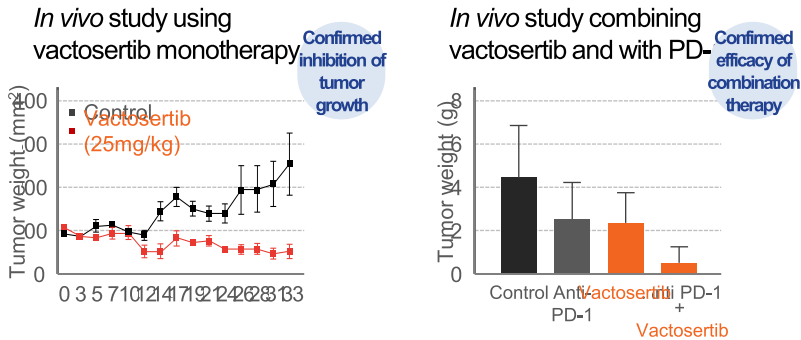
\* Empty box means "0"

- Among 105 evaluable patients, rash, headache and decreased appetite were the most frequent treatment emergent adverse events (TEAEs). All were manageable and no fatal TEAEs were observed in any cohort.

### 3. Vactosertib\_Market potential as a blockbuster drug candidate

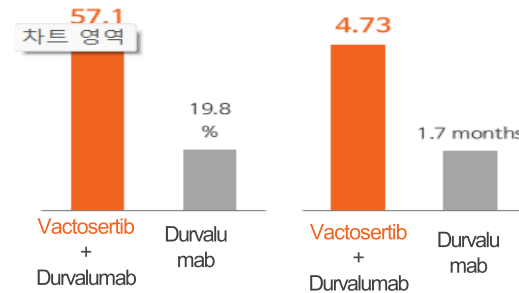
Promising efficacy results serve as a platform that indicates various partnering opportunities in other indications

#### Pre-clinical results in gastric cancer model



#### Combination trials in NSCLC (P2 interim results)

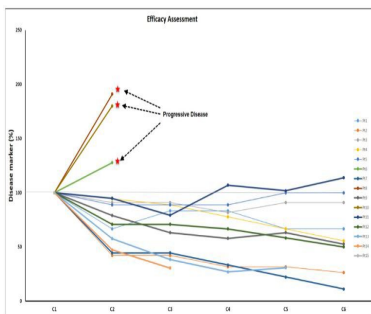
Objective Response Rate (ORR)    Progression Free Survival (PFS)



Source: AstraZeneca DCR, ORR data, Antonia et al. Journal of Clinical Oncology 2017;35:9085-9085  
Note: A study directly comparing the combination therapy vs. Imfinzi was not conducted.

#### Multiple Myeloma : 80% progression Free survival Rate

Preliminary clinical results in patients with multiple myeloma who have progressed on conventional therapies (such as Pomalyst and dexamethasone)

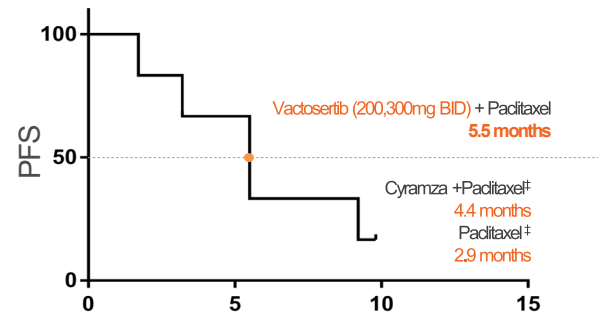


Metrics	Vactosertib combo	ELOT +Pd	DARA +Pd	POM+ DEXA(Pd)	POM
6M Progression free survival (PFS)	80%	62%	58%	40%	20%

Note: Daratumumab historical data source: Chari et al. (2017) Blood. 130:974

#### Gastric Tumor Combination Therapy

Progression Free Survival (PFS)



Source : <sup>†</sup> MedPacto ESMO 2020 Poster, <sup>‡</sup>Lancet Oncol. 2014 Oct;15(11):1224-35.  
Note : A study directly comparing the combination therapy vs. Cyramza was not conducted. Results from phase 1b stage

Potentially ground-breaking treatment for osteosarcoma,  
an area that has shown no significant advancement in SoC for decades

#### Highly debilitating pediatric cancer



**~ 1,000**

Incidence in US every year, of

which about half, **400**

impacts **children and teenagers**



**Prognosis** of recurrent disease is poor  
- **long-term post relapse survival of**

**<20%**

#### SoC unchanged for >50 years



**1970 ~**

Same combination of drugs used to treat for last half century (MAPIE)

\* MAPIE: Cocktail chemotherapy treatment



There is **no accepted standard regimen** for **second-line therapy for recurrent** patients

### Compassionate use in the US, now in US FDA/MFDS trials



- Recurrent osteosarcoma with brain and lung metastasis
- Vactosertib monotherapy for 16 months
- **Out of hospice, at school, remains free of metastasis (as at March 2023)**



### 3. Vactosertib\_Monotherapy in Osteosarcoma

Received recognition from the U.S. FDA for Vactosertib's capacity to address critical medical needs, accelerate development, and underscore innovation within the field of pediatric oncology

**Orphan Drug Designation (2021.08)**

**Rare Pediatric Drug Designation (2022.09)**

**Fast Track Designation (2023.01)**

**Orphan Medicinal Product Designation (2023.07)**

**Initiate Clinical Trials in US/KR**

**Study to Assess Safety and Efficacy of Vactosertib in Adolescents and Adults With Recurrent, Refractory or Progressive Osteosarcoma**  
 [NCT 05588648] / Phase 1/2 / Estimated Enrollment: 48, FPI : April

	Orphan Drug Designation; ODD	Rare Pediatric Designation; R PDD
Benefits	<ul style="list-style-type: none"> <li>Exemption from user fees</li> <li>Reduced fees for regulatory activities</li> <li>Tax credits for qualified clinical trials</li> <li>Potential seven years of marketing exclusivity after approval</li> </ul>	<ul style="list-style-type: none"> <li>Additional incentives for obtaining FDA approval of such products beyond the incentives offered by the orphan drug designation (ODD) program</li> <li>Speed the review and potential approval of treatments</li> <li>PRV(priority review voucher)</li> </ul>
	Fast Track Designation ; FTD	Breakthrough Therapy Designation ; BTD
Benefits	<ul style="list-style-type: none"> <li>More frequent meetings with FDA to discuss the drug's development plan and ensure collection of appropriate data needed to support drug approval</li> <li>More frequent written communication from FDA</li> <li>Eligibility for Accelerated Approval and Priority Review</li> <li>Rolling Review</li> </ul>	<ul style="list-style-type: none"> <li>All Fast Track designation features</li> <li>Intensive guidance on an efficient drug development program, beginning as early as Phase 1</li> <li>Organizational commitment involving senior managers</li> </ul>

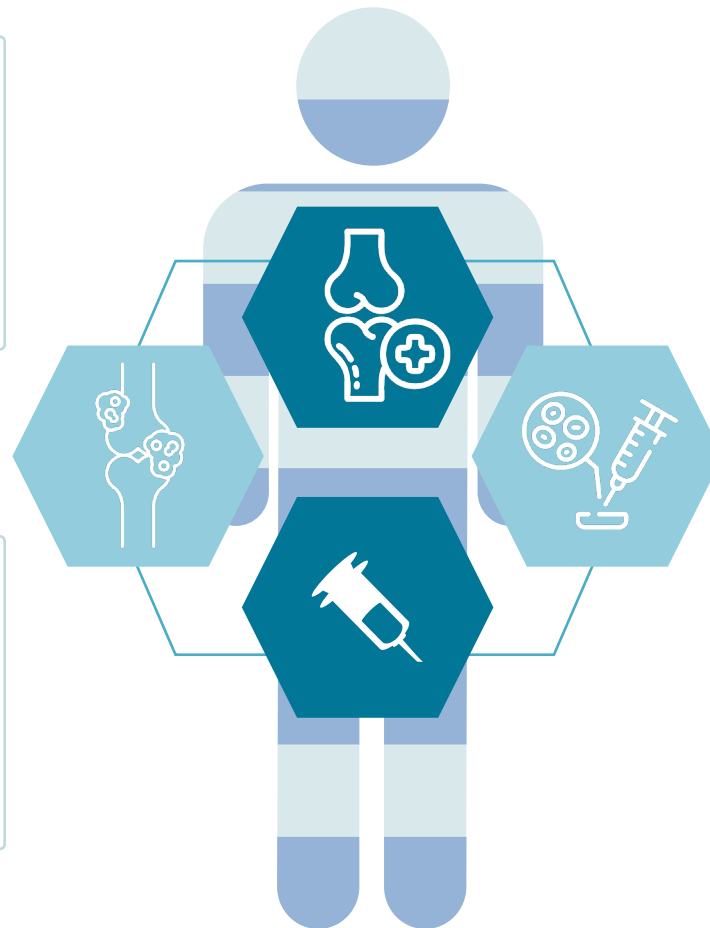
An innovative, First-in-class biologics program in the preclinical stage with remarkable data to date

### Novel Dual-Target Protein

- MP2021 is a First-in-class pipeline that is discovered by MedPacto
- MP2021 is expected to inhibit macrophage and osteoclast.

### Various Inflammatory diseases and bone-related cancers

Potentially safe and effective treatment for various inflammatory diseases and bone-related diseases



### KDDF granted

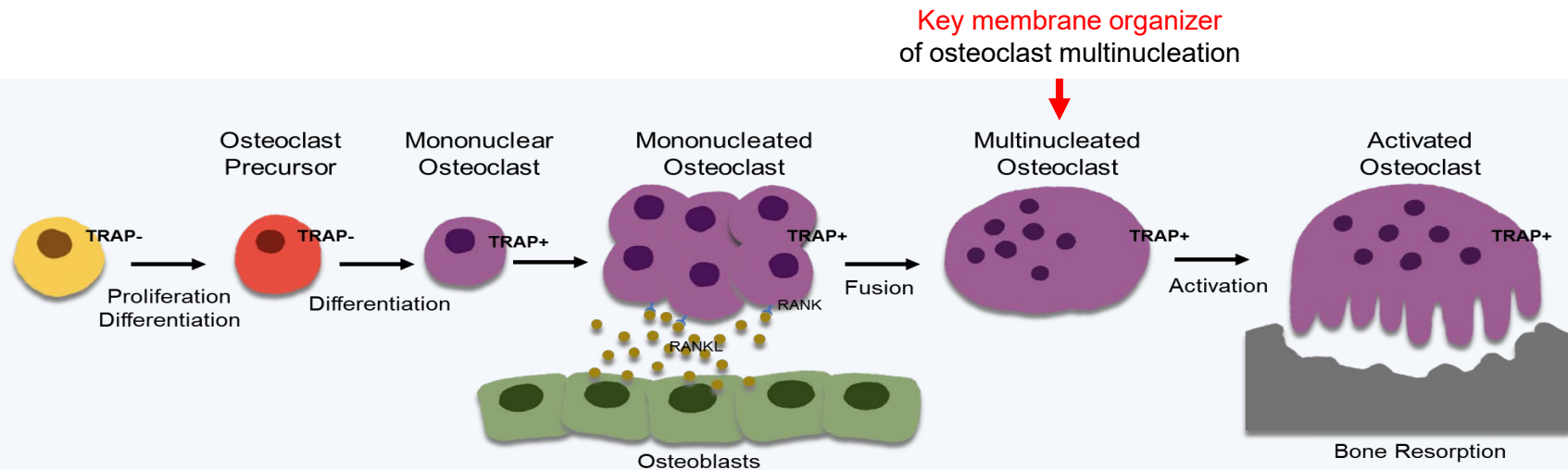
- Financial, regulatory, and collaborative support by the government
- Enhanced credibility opens doors to opportunities in the pharmaceutical and biotech sectors.

### Strong Preclinical Data

- Rheumatoid arthritis potential – MP2021 significantly reduces expression of inflammatory cytokines in an autoimmune model (collagen-induced arthritis)
- MP2021 significantly inhibits ovariectomy-induced bone loss, signifying its potential as a therapeutic

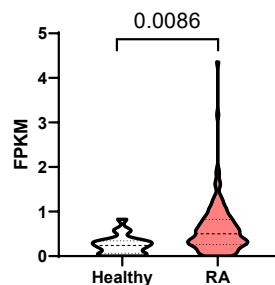


By effectively inhibiting the formation of multinucleated osteoclasts in the later stage of the multinucleation process, MP2021 is positioned to demonstrate remarkable safety and efficacy



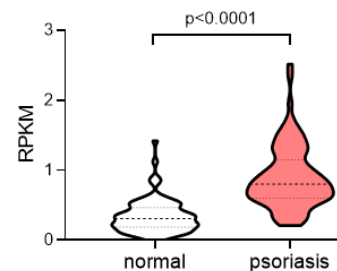
### Public Patient Data

#### Rheumatoid arthritis



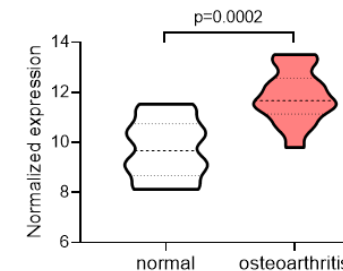
GSE120178

#### Psoriasis



GSE54456

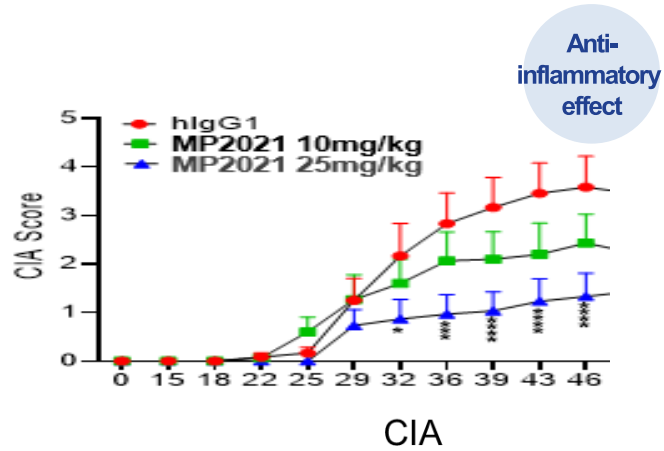
#### Osteoarthritis



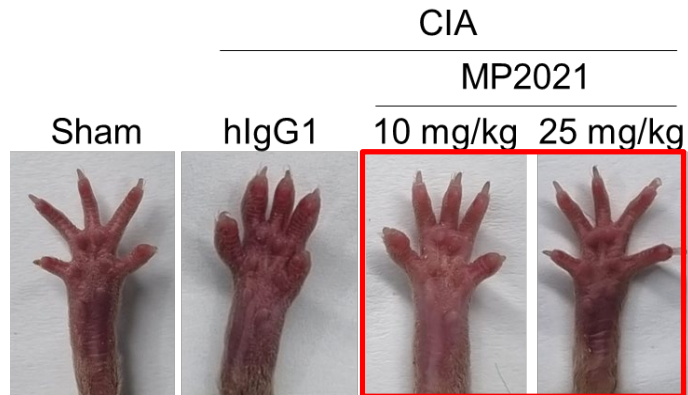
accession E-MTAB-5564

MP2021 has demonstrated its efficacy in restoring bone and cartilage in the Collagen-induced Arthritis Model by increasing bone mass and density

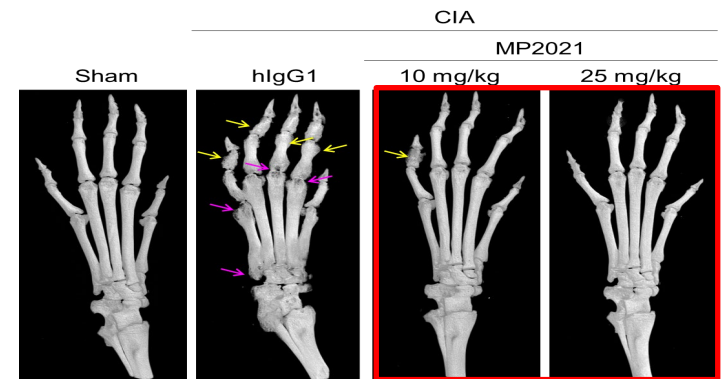
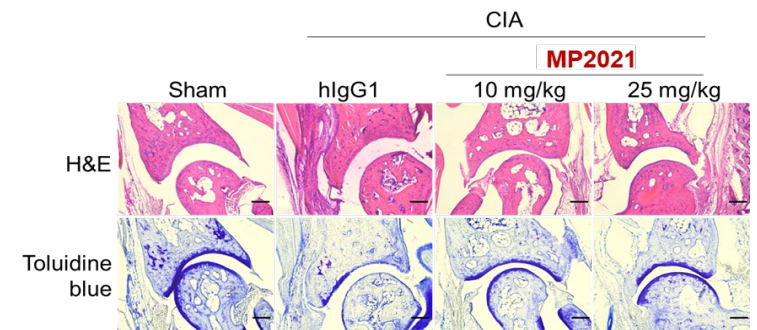
Collagen-induced Arthritis Model  
MP2021 Monotherapy result



Anti-inflammatory effect



Collagen-induced Arthritis Model  
Tissue staining & Micro CT Result



## 5. Clinical Advisory Board

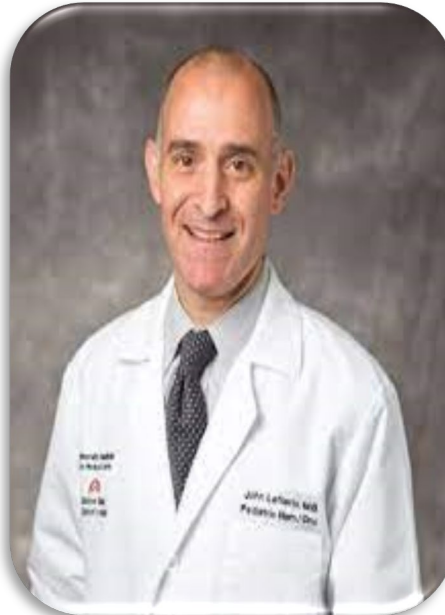
Consisting of leading global experts from prestigious academic institutions, our team is dedicated to shaping global clinical strategy and driving business development

**Dr. Greg Licholai, M.D., MBA**



- Former President of Rare Diseases at Moderna Therapeutics and Vice President at McKinsey & Co.
- Currently Chief Medical & Innovation Officer at ICON plc.
- Distinguished faculty member at Harvard Business School and Yale School of Management.

**Dr. John Letterio, M.D.**



- Currently Director at the Angie Fowler Adolescent & Young Adult cancer Institute.
- Associate Director at the Seidman Cancer Center, University Hospitals, Case Western Reserve University.

**Dr. Hyun Bae, M.D.**



- Currently a board-certified orthopedic surgeon specializing in spine surgery
- Practices at the Spine Center, Cedars-Sinai Medical Center, and the Spine Institute in Santa Monica, California.

**Dr. Issac Kim, M.D.**



- A renowned urology oncologist
- Currently, Professor of Urology and Chair at Yale University School of Medicine
- Previously, completed a urologic oncology research fellowship at the National Cancer Institute and served as the Executive Director of the Cancer Institute.



Thank You