FY2023 1st Half Business Results
November 15, 2023

Perseus Proteomics Inc.

(Securities code: 4882)

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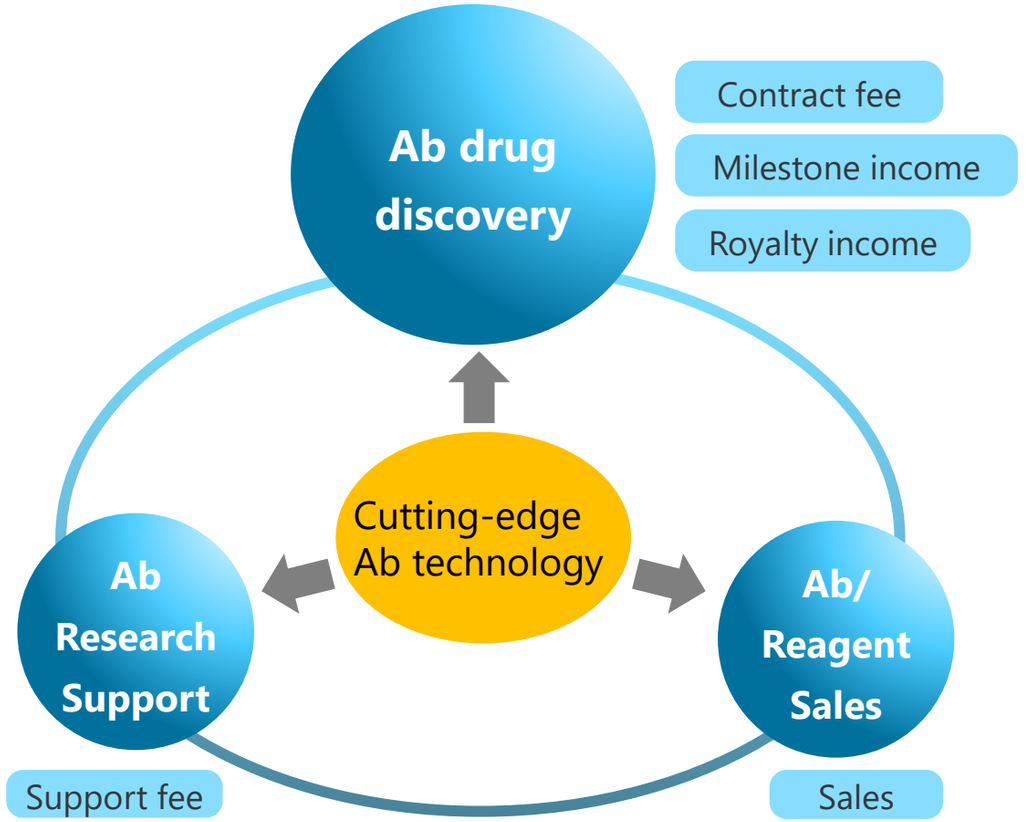
01 About Perseus Proteomics

Company outline

We contribute to global healthcare with cutting-edge antibody technology

Company name	Perseus Proteomics Inc.
Established	February 2001
Business	● Antibody (Ab) drug discovery ● Ab research support ● Ab/reagent sales
Securities code	4882
Office	HQ/Laboratory: 30-1 Nihonbashi-Hakozakicho, Chuo-ku, Tokyo, Japan Nagoya Laboratory: 2-22-8 Chikusa, Chikusa-ku, Nagoya-shi, Aichi, Japan
Capital	1,939 million yen*
Employee	27*

*as of Sep.30, 2023



02

Progress of FY2023 Plans

Progress of FY2023 plans

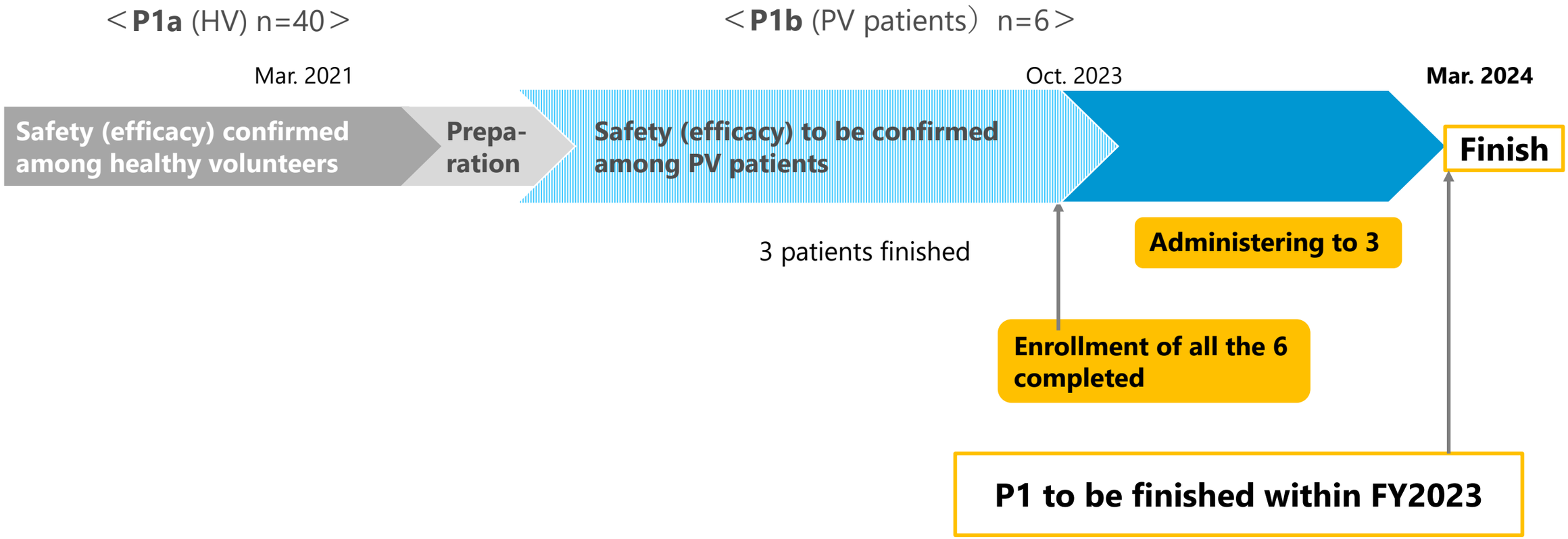
FY2023 Plans	Progress of 1st half
1 **PPMX-T003: Finish Polycythemia vera (PV) P1, licensing out in FY2024**	Completed 6 PV patients enrollment Initially administered to 2 new patients
2 **PPMX-T003: Promote Aggressive NK-Cell Leukemia (ANKL) investigator-led P1/2**	Initially administered to 2 ANKL patients
3 **PPMX-T002: Formulate development plan for licensing out in FY2024**	Studying development plan with plural licensing candidates
4 **PPMX-T004: Narrow down linker/drug Start preliminary toxicity tests**	Confirmed efficacy in animal model Conducting preliminary toxicity tests

Pipeline

Code	Indication	R&D		Clinical trial			Approval	Market size（2024 forecast）
		Drug discovery	Non-clinical	P1	P2	P3		
PPMX-T003	PV				～FY2024 License-out			USD 1,923 M＊1
	ANKL							—
PPMX-T002	Solid tumor (ovarian cancer, etc.)			RI Ab（Y）	～FY2024 License-out			USD 5,430 M＊2
				RI Ab（Ac）				
PPMX-T004	Solid tumor (ovarian cancer, etc.)			ADC				USD 5,430 M＊2
				ADC				

＊1　Total of seven main markets (JP, US, EU5), Source: Global Information “Polycythemia Vera Treatment: Besremi, Jakafi, and Rusfertide Analysis”
＊2　Source: Evaluate Pharma Ovarian Cancer WW Market Value (2021), ovarian cancer only

1 PPMX-T003: PV
Patients enrollment & initial administration to 2 patients completed in P1



1 PPMX-T003: PV
PPMX-T003 attracts attention at 65th ASH meeting/exposition, etc.

Interim report of P1 Presentation

- **Safety and efficacy confirmed** in all the 3 patients

JP	May 27		118th **JSH Kinki Association**
JP	Jul. 21-22		13th JSH International Symposium 2023
JP	Dec. 1	NEW	**2nd Antibody Society of Japan Meeting**
US	Dec. 9 -12	NEW	**65th ASH Annual Meeting and Exposition**

Adopted as presentation at the world largest hematology society meeting/exposition

URL: https://ash.confex.com/ash/2023/webprogram/Paper174193.html

Title: Interim Phase I Safety Study of Anti-Transferrin Receptor Antibody (PPMX-T003) in Patients with Polycythemia Vera

1 PPMX-T003 market: Position & superiority among competitive PV drugs

PV characteristics: increase in RBC ⇒ blood viscosity↑ ⇒ thrombosis

High risk
Low risk

: 60~ or thrombosis in the past (certain level of AEs are tolerable)
: under 60, no history of thrombosis

	Inhibits hematopoiesis	Decrease in internal iron amount	Inhibits RBC generation only
Infusion/ injection	③ Interferon / Interferon Every 2 weeks	① Phlebotomy / Phlebotomy	⑤ PPMX-T003 / PPMX-T003 Every 3 weeks
Oral	② JAK2 inhibitor DNA synthesis inhibitor	The more RBCs decrease, the severer adverse effects appear.	

Disease progress

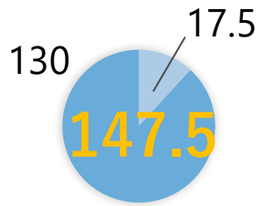
New drug

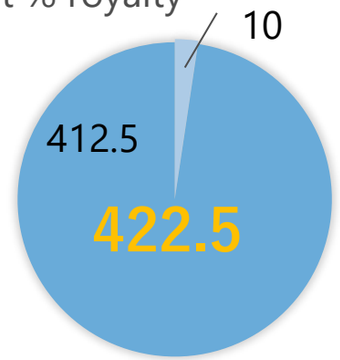
Adverse effects ×

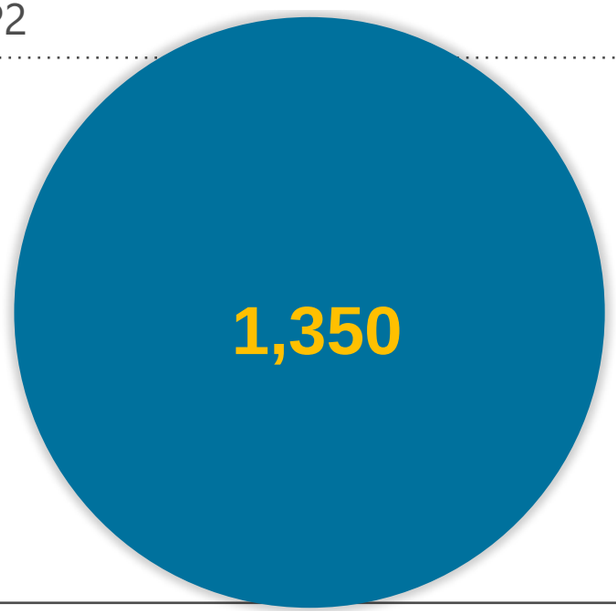
△QOL lowers ○

1 PPMX-T003 market: Recent PV drug related license-out/acquisition cases (hot market)

Category	License-out＊1（Aug. 2023）	License-out＊2（Jan. 2023）	Acquisition＊3（Nov. 2022）
Licensee/ Acquiree	Alnylam Pharmaceuticals (US)	Mabwell Therapeutics（CN）	Imago BioSciences (US)
Licenser/ Acquirer	Agios Pharmaceuticals (US)	Disc Medicine (US)	Merck & Co.（US）
Indication	PV	PV, beta-thalassemia	Myeloproliferative neoplasms (including PV)
Stage	Pre-clinical	P1	P2
Contract fee (million USD）	＋Commercial milestone ＋Royalty	＋2-digit % royalty	







Contract fee　Development (&commercial) milestone

＊1 Agios Pharmaceuticals, 2023-8-3 “Agios Announces Exclusive Worldwide License Agreement with Alnylam for Novel siRNA for the Potential Treatment of Polycythemia Vera”
＊2 Disc Medicine, 2023-1-20 “Disc Medicine Announces Exclusive Licensing Agreement with Mabwell Therapeutics for Novel Anti-TMPRSS6 Monoclonal Antibodies to Modulate Iron Homeostasis”
＊3 Merck, 2022-11-21 “Merck to Acquire Imago BioSciences, Inc.”

2 PPMX-T003: ANKL
Ultra-rare disease; causes to be discovered and treatment to be established

What's ANKL?

- **Poor prognosis**

  50% survival period = 58 days (about 2 months)

- **Ultra rare disease**
  - 13 cases in Japan in 2020
  - Many cases in east Asia and Latin America
  - Cases may increase when advances in medical technology will enable easier diagnosis
- **Many cases in AYA generation** (age 15 to 39) and **40s**
- **No effective medical drug available**

Subsidy from AMED*

FY2022:	58M yen	Received
FY2023:	107M yen	+7 increase approved
FY2024:	100M yen	
Subsidy (max) total:	265M yen	

* Through Project Promoting Support for Drug Discovery Support Program for Orphan drug prior to the Designation

2 PPMX-T003: ANKL
Investigator-led P1/2 (n=7) started in Mar. 2023, 2 patients administered

2022/03 2023/09 2025/03

ANKL basic research (Tokai Uni) → **Development** (Tokai Uni+PPMX) → **Investigator-led P1/2 clinical trial** (Hiroshima Uni+PPMX)

PPMX-T003

Clinical trial notification submitted (Prof. Ando at Hiroshima Uni)

2 patients administered



P1/2 finishes

Approval of world-first effective ANKL drug Orphan drug application

- **7 clinical trial locations**
- **Nationwide coverage**

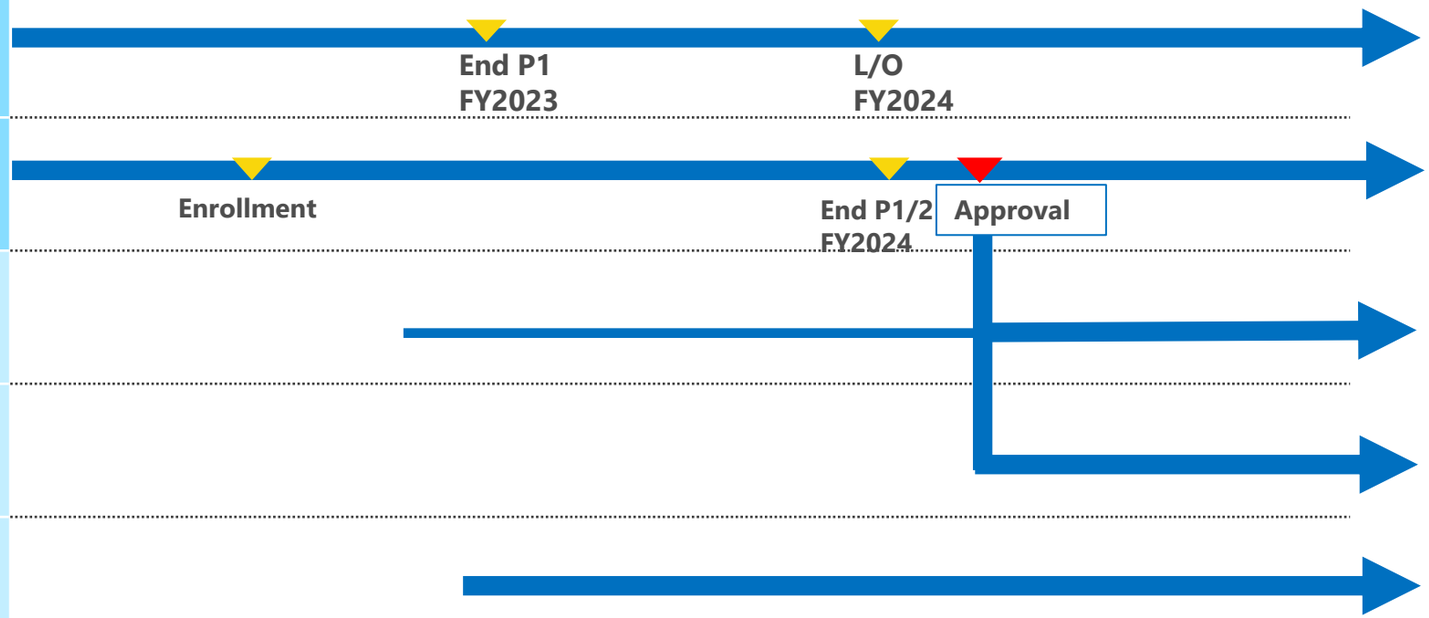
- **Clinical trial information**

jRCT
https://jrct.niph.go.jp/en-latest-detail/jRCT2061230008

2 PPMX-T003: ANKL
Expand indications of T003 through efficacy confirmation in cancer field

Indication	No. of patients	Stage
PV	300,000*1	P1 (patients)
ANKL	-	Investigator-led P1/2
AML	180,000*2	Joint research w/academia
Lymphoma	620,000*3	Joint research w/academia
Solid tumor	18.1 million*4	Joint research w/academia

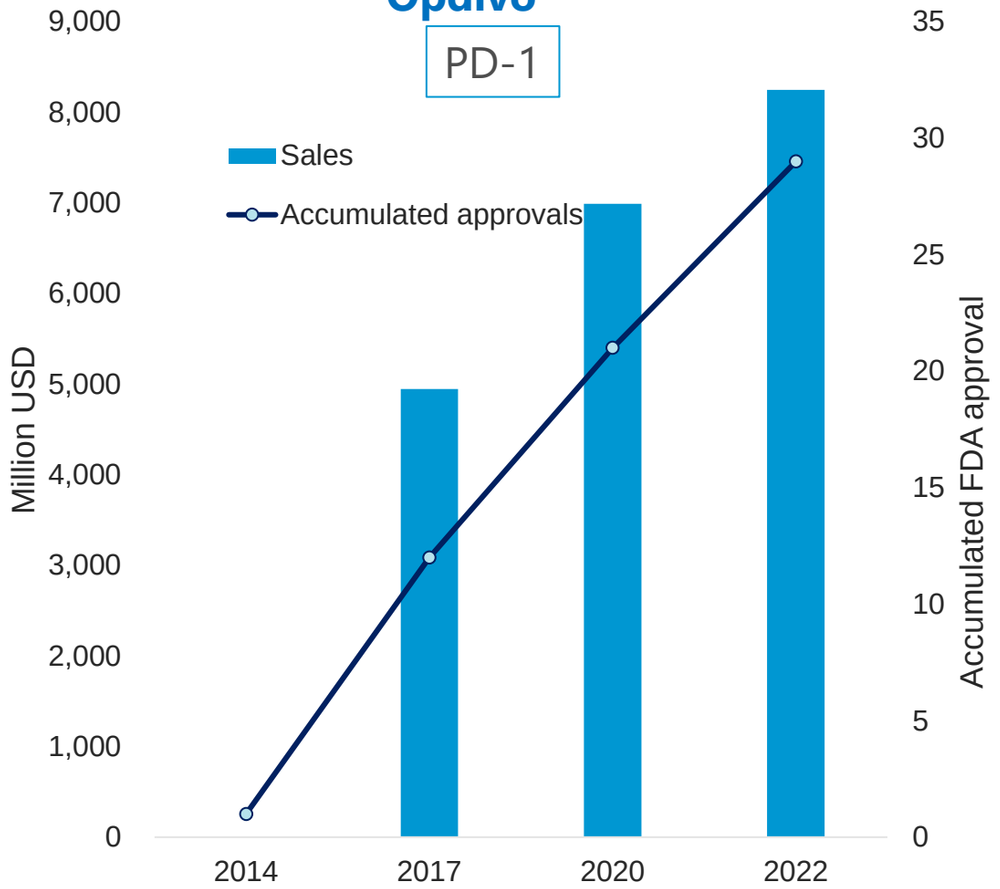
Development timeline image



Source： *1 Global Information “Polycythemia Vera Treatment: Besremi, Jakafi, and Rusfertide Analysis”
*2 Prepared by the Company based on WHO data and “Blood Reviews” Volume 36, Epidemiology of acute myeloid leukemia: Recent progress and enduring challenges”
*3 WHO *4: World Cancer Research Fund International (2020)

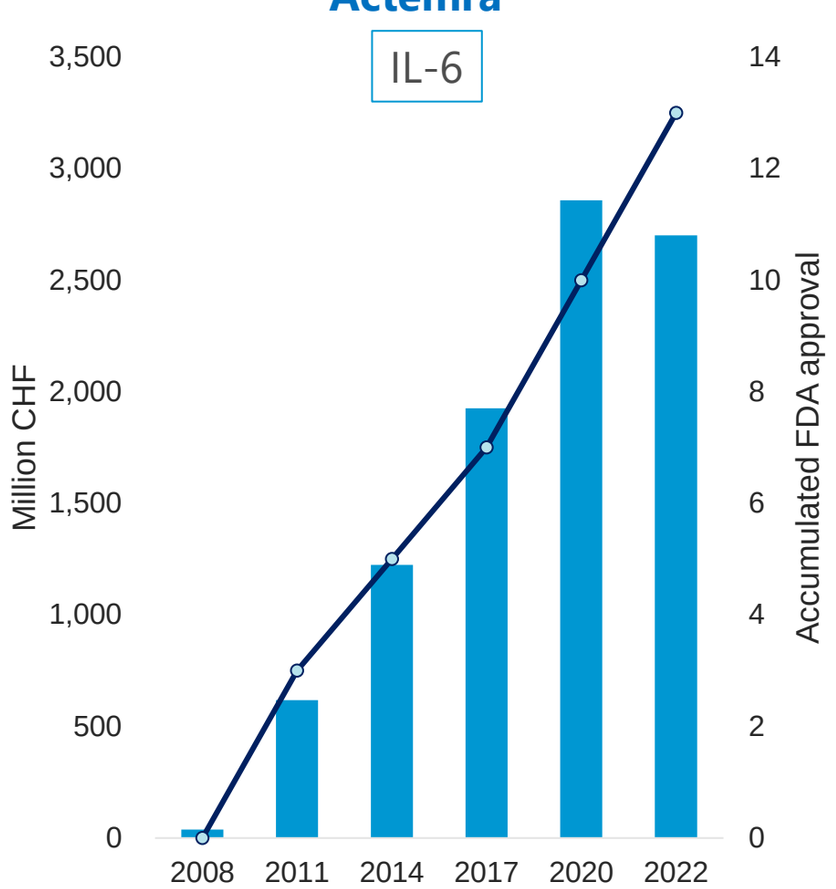
From orphan drugs to block busters
Examples of Japan-oriented Ab medical drugs

Opdivo*1



Melanoma: JP (1/100,000) → Non-small cell lung cancer kidney cancer, stomach cancer, Hodgkin lymphoma, etc.

Actemra*1

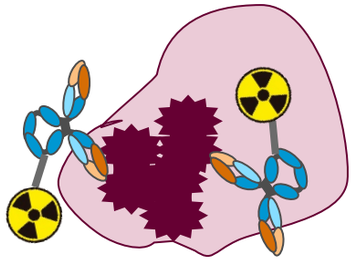


Castleman disease: JP*2 (1,500/yr in JP) → articular rheumatism, etc.

＊1 Prepared by the Company based on IR materials of Bristol Meyers Squibb and Roche.
＊2 Sales started by Chugai Pharmaceuticals in 2005 in Japan.

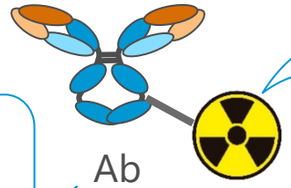
3 PPMX-T002: Function of RI labelled Ab and structure of new PPMX-T002

1 Function of RI labelled Ab



PPMX-T002 Ab accumulates on CDH3 on cancer cells and RI kills cancer cells

2 Structure of new PPMX-T002



Confirmed accumulation on cancer cells (in P1 expansion in USA)

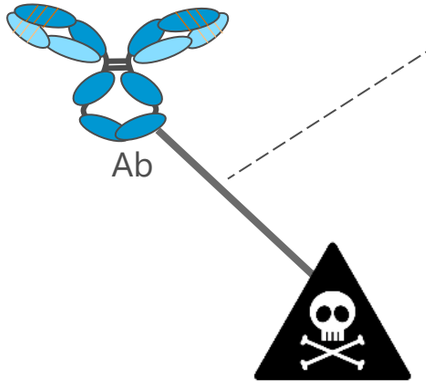
^{90}Y(β emitter)⇒**^{225}Ac (α emitter)** to improve efficacy

3 Achievements in first half and future strategy

Q1-Q2	Q3-Q4	FY2024
Study development strategy w/plural licensing candidates	Determine a licensing partner	License out

4 PPMX-T004: Under development as a new ADC

1 Development plan



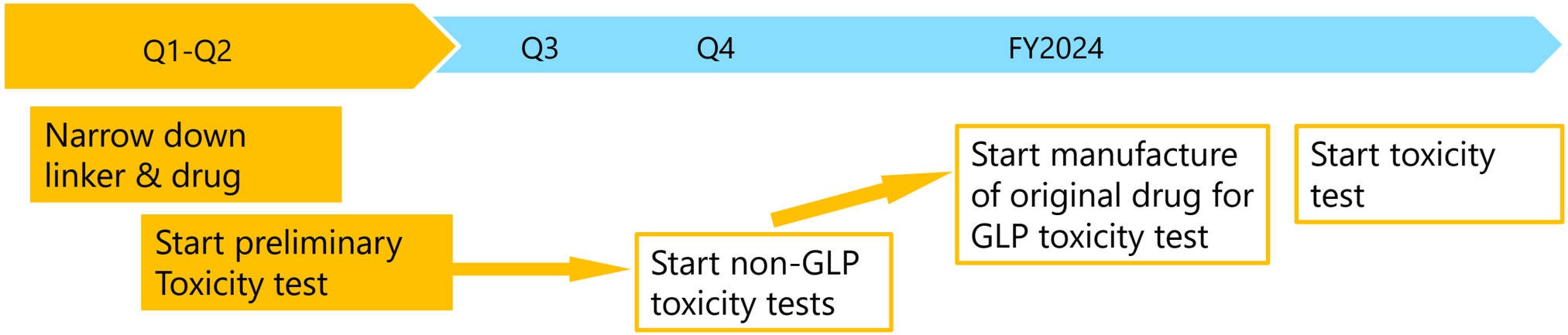
Linker

×

Small molecule anti-cancer drug

- High stability in blood
- Separated at the intended timing to release anti-cancer drug
- Small molecule anti-cancer drug that matches antibody characteristics

2 Achievements in first half and future strategy



03 Other Topics

Ab/reagent sales and Ab research support
Aim to improve revenues by introducing new products/service utilizing drug discovery technology

Ab/reagent sales

Introduced 3 new products

- for **ADC** research
  - Anti-DM1 Ab
- for **RIT** research
  - Anti-DOTA Ab
- for **disease** research
  - Anti-mAQP4 Ab

Ab research support

Expand service menu utilizing single cell cloning technology

- Single cell gene analysis
- Retrieval of rare cells
- Cell cloning after inserting gene

Termination of development of super neutralizing antibody “UT28K” for COVID-19

Unexpected evolution of BA.5 and variants afterwards

- Regions that were not thought to undergo mutation **mutated**
- Newly mutated regions **acquired infectivity**

Ab generation technology of Toyama University

Obtain Ab quickly from recovered patients

Clues for innovative drug in Abs extracted from recovered patients

PPMX : considers the technology as an important platform for obtaining Ab

04 FY2023 1st Half Business Results

FY2023 1st half business results
Profit & Loss highlights

(million yen)

	FY2022 1st half	FY2023 1st half	FY2023 Forecast
Sales	**47**	**50**	100
Gross profit	44	45	91
SG & A	351	489	1,082
R&D cost	214	303	752
Other	137	186	330
Operating income	**(306)**	**(444)**	(991)
Ordinary income	**(288)**	**(429)**	(991)
Extraordinary loss	73	203	192
Net income	**(363)**	**(633)**	(1,185)

Ab research support
Ab/reagent sales

PPMX-T003 clinical trial
Pre-clinical tests of PPMX-T004

Impairment loss due to capex and head office relocation

- Sales: Almost as planned
- R&D cost: PPMX-T003 PV P1 and pre-clinical tests of PPMX-T004, etc.

FY2023 1st half business results
Balance Sheet highlights

(million yen)

Assets	2023/3/31	2023/3/31
Cash & deposits	2,444	1,866
Total current assets	2,514	1,946
Non-current assets	51	42
Total assets	2,566	1,989

Liabilities	2023/3/31	2023/3/31
Current liabilities	111	96
Non-current liabilities	58	108
Total liabilities	170	205
Total net assets	2,396	1,783
Total liabilities and net assets	2,566	1,989

- Cash & deposits: decrease mainly due to R&D costs and capex
- Non-current liabilities: long-term deposits due to adoption of PPMX-T003 (ANKL therapeutic drug development) as AMED program
- Capital ratio: 87.0%

05 Future Goals and Plans to Achieve them

Measures to implement our growth strategy



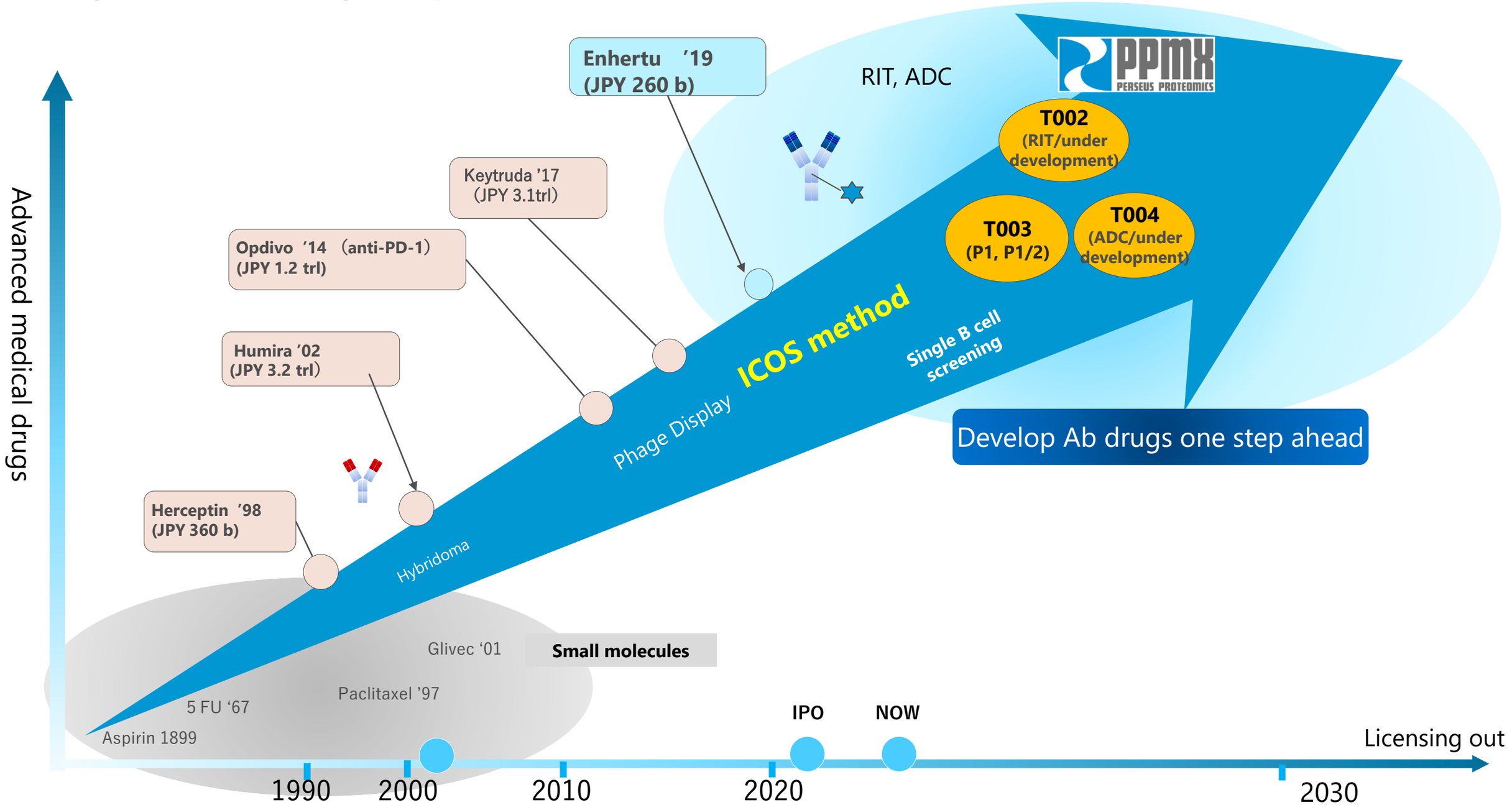
Develop, expand indication, and promote licensing out of current pipeline
PPMX-T003（PV, ANKL), PPMX-T002, PPMX-T004

Core technology to generate next pipeline continuously
Improve phage libraries, establish core technology

Enhance R&D structure
- **Proactively recruit researchers**
- **Improve research environment further**

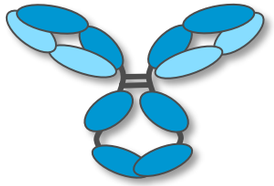
Make profits from licensing out of pipeline
Raise market evaluation through communication w/investors
Promote growth through utilization of capital market

Bring more Ab drugs to patients



Appendix

Antibody drugs

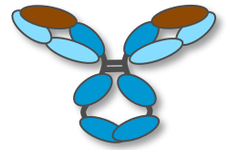


Antibody

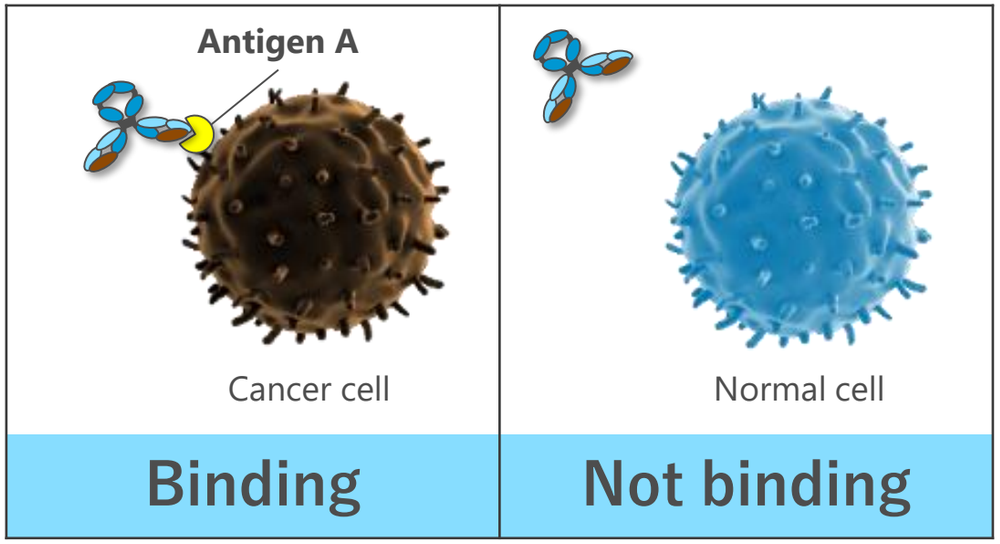
- Remove alien objects inside body
- Innate immune system
- Act on specific antigens (targets) only

⇒ ideal molecule targeting drug with few adverse effects

→ **High safety**



Antibody A

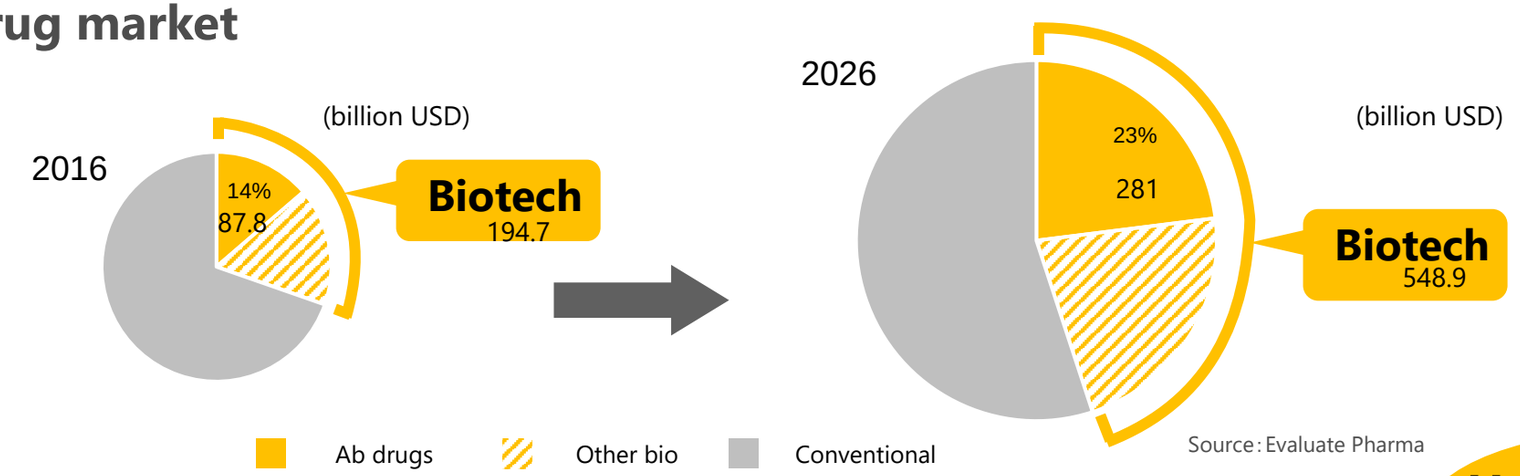


→ **Ab drug**

Medical drug consisting of antibody designed to act on disease regions only

Antibody drug market

Global Ab drug market



Source: Evaluate Pharma

Half of top 10 Are Abs

Medical drug sales top 10 in 2022 (excludes COVID-19 vaccinations)

	Name	Sales (million USD)	Indication/Type
1	**Humira**	**21,615**	Rheumatism/**Ab**
2	Keytruda	20,937	Cancer/**Ab**
3	Paxlovid	18,933	COVID-19/small molecule
4	Eliquis	18,269	Anticoagulation/small molecule
5	Biktarvy	10,390	HIV／small molecule

	Name	Sales (million USD)	Indication/Type
6	Stelara	10,220	Psoriasis/**Ab**
7	Eylea	10,198	Age-related Macular Degeneration/Bio
8	Revlimid	9,978	Multiple myeloma/small molecule
9	Opdivo	9,294	Cancer/**Ab**
10	Dupixent	8,741	Atopic dermatitis／**Ab**

Source: Prepared by the Company Based on information as of Apr. 25, 2023 "Nikkei Biotech ONLINE" https://bizboard.nikkeibp.co.jp/bp_bto/atcl/column/16/011900001/23/04/21/00341/

PPMX-T003 market:
Recent PV drug related license-out/acquisition cases (details)

(million USD)

		R&D	P1	P2	P3
Stage		Pre-clinical	P1	P2	
Type		License-out	License-out	Acquisition	
Contract fee	Upfront	17.5	10		
	Development/ Commercial milestone	130	412.5		
	Royalty	YES	YES (2-digit %)		
	TOTAL	147.5 (＋royalty)	422.5 (＋royalty)	1,350	
Licenser/Acquirer		Alnylam Pharmaceuticals (US)	Mabwell Therapeutics (CN)	Merck & Co. (US)	
Licensee/Acquiree		Agios Pharmaceuticals(US)	Disc Medicine(US)	Imago BioSciences (US)	
Development		siRNA drug candidate targeting TMPRESS6	Anti-TMPRSS 6 Ab, Abs w/same MoA	Myeloproliferative neoplasms (including PV, etc.)	
Contract type		Exclusive commercial rights	Exclusive commercial rights		
Region		WW	US, EU, and other regions excluding CN and Southeast Asia		
Announced		2023/8/3	2023/1/20	2022/11/21	

Focus of Ab drug discovery competition
To the top of Ab biotech ventures utilizing superiority in technology

Select theme
Needs
Market superiority

Obtain best Ab
- Collect as many as Abs
- Select the best Ab efficiently

Valuation of pipeline determined at this stage

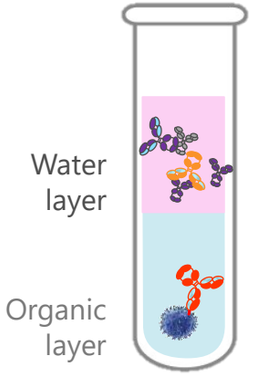
Clinical development → **License-out**
Most important for business progress
Centre of business results report

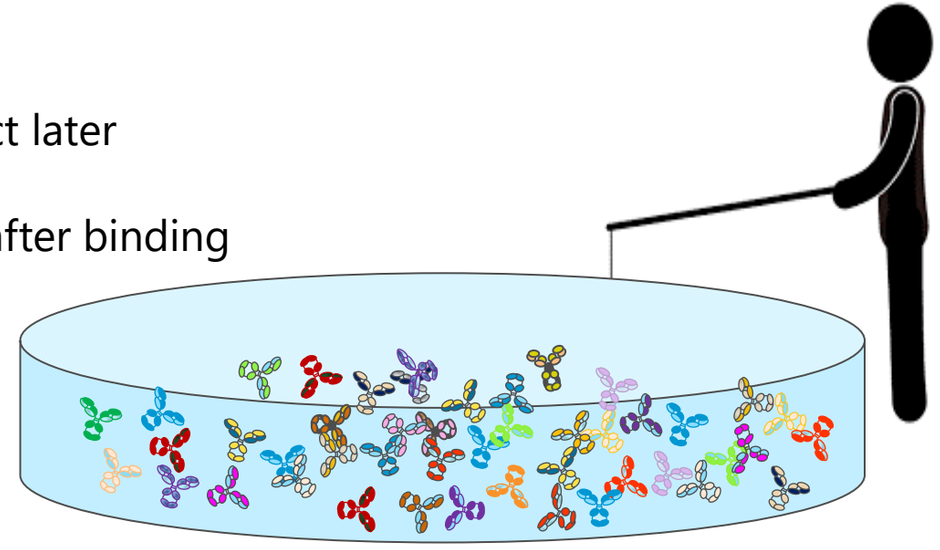
Performance evaluated by progress of development

Methods to collect Abs (Utilize 3 basic Ab obtaining methods and improve continuously)
1) Recovered patients 2) Animal immune 3) Phage display method

Methods to select
ICOS method using cells
Individual target fishing vs. Fishing with a fishing net to select later

Select Ab by not only binding affinity but also functionality after binding
(enter inside of cells, bind receptors to block others, etc.)

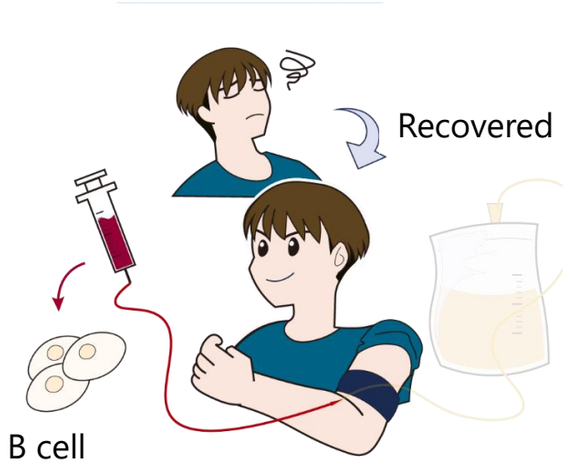




Cover the methods to create Ab drugs and pursue cutting-edge technology

1 Recovered patients

Disease essence and clues for medical drugs

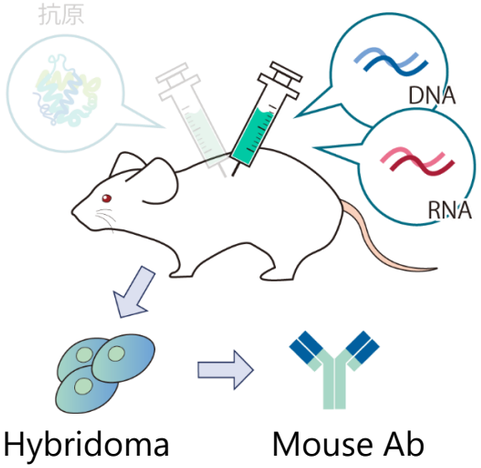


Generate Abs from one cell in blood

Single cell cloning service

2 Animal immune

Exquisite for gene manipulating drug discovery



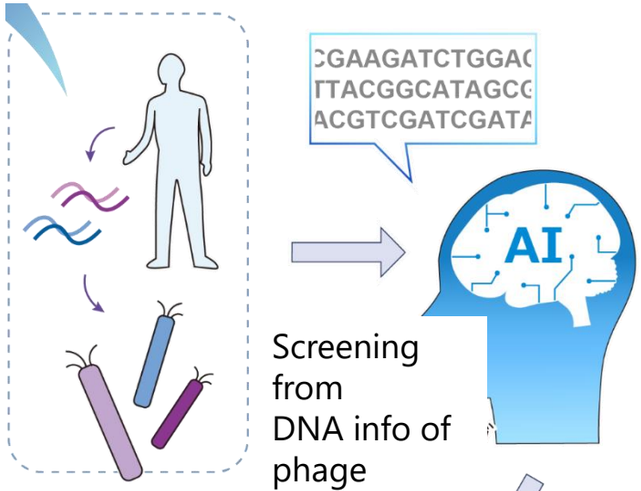
Humanization

Humanize murine Abs

Cell cloning service after inserting genes

3 Phage display method

Combine sequence info of NGS analysis and actual data



AI selects targeted Ab from library

Utilize our numerous Abs and actual data to AI



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https://www.ppmx.com/en/

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