R&D 説明会

June 8, 2026

ONO PHARMA

注意事項

この資料に記載されている業績見通し等の将来に関する記述は、当社が現在入手している情報及び合理的であると判断する一定の前提に基づいており、実際の業績等は様々な要因により、大きく異なる可能性があります。

以下に、事業展開上のリスク要因となる可能性があると考えられる主な事項を挙げますが、これらに限定されるものではありません。

（ｉ） 新製品開発の失敗
（ii） 医療保険制度の改革による事業環境の変化
（iii） 競合品や後発品の影響により、期待した成果を得られない可能性
（iv） 第三者による知的財産の侵害等
（ｖ） 自然災害や火災等で、生産の停滞・遅延発生による製品供給の滞り
（vi） 市販後の医薬品における新たな副作用の発現
（vii） 為替レートの変動や金利動向

また、この資料には医薬品（開発中のものを含む）に関する情報が含まれていますが、宣伝広告、医学的アドバイスを目的としているものではありません。

本日のアジェンダ

オープニング

POC試験のデータ詳細

- ONO-4578（ASCO2026）
- ONO-2808（7th 世界パーキンソン病学会）

中枢神経領域における創薬活動

滝野　十一

代表取締役社長　COO

岡本　達也

執行役員　開発本部長

勝又　清至

執行役員　研究本部長

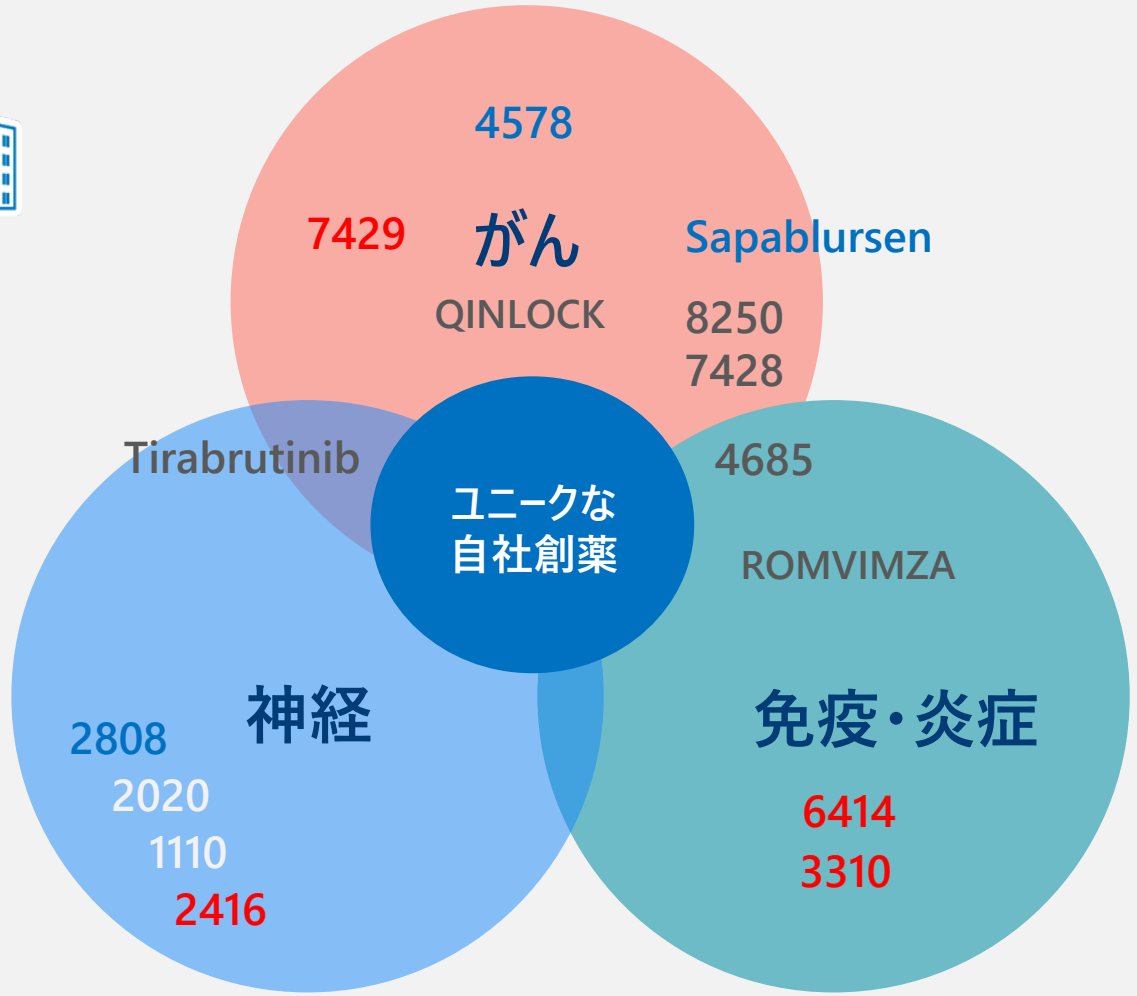
3つの疾患領域へのコミットメント

2026年5月8日現在

・3つのグローバルP3試験の開始や、7つのPOC試験*のデータリードアウトを予定
・がん、免疫・炎症、神経の重点領域において新たな4つのパイプラインがP1試験の開始

オープン
イノベーション

ライセンス



グローバルP3試験の開始
POC試験のデータ取得
P1試験の開始

7つのPOC試験*：ONO-2020（2つ）とONO-1110（5つ）

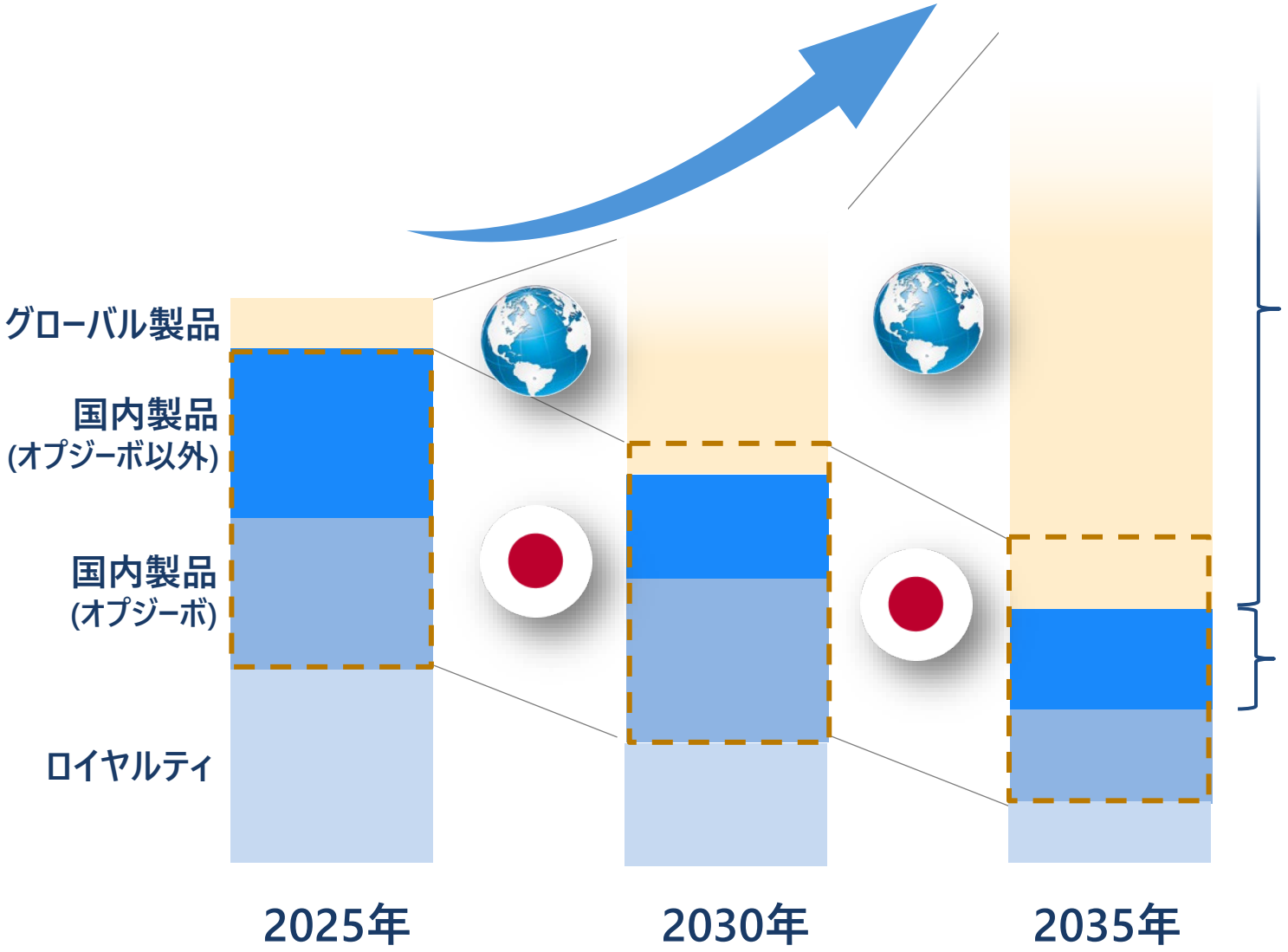
新製品の上市予定

2026年6月8日現在

	2024年度	2025年度	2026年度	2027年度	2028年度～
欧米	QINLOCK® 消化管間質腫瘍4L	ROMVIMZA® 腱滑膜巨細胞腫	Tirabrutinib ONO-4059 中枢神経系原発リンパ腫	QINLOCK® 消化管間質腫瘍2L KIT Exon 11+17/18	日本を含めグローバルに展開 Sapablursen ONO-0530 真性多血症 ONO-4685 T細胞リンパ腫/自己免疫疾患 ONO-4578 胃がん1L（オプジーボ併用） ROMVIMZA® 慢性移植片対宿主病 ONO-2808 多系統萎縮症
日本	オプジーボ MSI-H 結腸・直腸がん1L セノバメート ONO-2017 てんかん部分発作 オプジーボ 肝細胞がん術後補助療法 ベレキシブル 天疱瘡 2L オプジーボ 皮下注製剤 オプジーボ 肝細胞がん 1L QINLOCK® 消化管間質腫瘍4L QINLOCK® 消化管間質腫瘍2L ROMVIMZA® 腱滑膜巨細胞腫 ビラフトビカプセル （FOLFOX） 結腸・直腸がん 1L povetacicept ONO-8531 IgA腎症/慢性腎症 Gel-One® ONO-5532 変形性関節症 セノバメート ONO-2017 てんかん部分発作 小児/ てんかん強直間代発作				

自社品　デサイフェラ　導入品

将来の売上成長イメージ



成長ドライバー

パイプライン	ピーク売上予想（億円）
グローバル	
QINLOCK (GIST)	500 – 700
ROMVIMZA (TGCT)	500 – 700
Tirabrutinib (PCNSL)	200 – 300
Sapablursen (真性多血症)	500 – 1,000
ONO-4578 (胃がん)	1,000 ~
ONO-2808 (多系統萎縮症)	1,000 ~
日本*	
Povetacicept	500 ~
Cenobamate	
Gel-One®	

*記載した3品以外も含んだ想定ピーク売上の合計

2026年5月8日現在

GIST: Gastrointestinal Stromal Tumor（消化管間質腫瘍）、TGCT: Tenosynovial Giant Cell Tumor（腱滑膜巨細胞腫）、PCNSL: Primary Central Nervous System Lymphoma（中枢神経系原発リンパ腫）

※イメージ図

2026年度の主なイベントスケジュール

	2026年度	
承認		ONO-4059 (VELEXBRU) PCNSL
承認	ONO-2017, セノバメート てんかん部分発作	QINLOCK GIST 4L
Phase3	ONO-0530, Sapablursen 真性多血症	ONO-4578 胃がん 1L
Phase3		ONO-2808 多系統萎縮症
Phase3		QINLOCK GIST 2L
Phase2	ONO-1110 帯状疱疹後神経痛	ONO-2020 アルツハイマー型認知症
Phase2	ONO-1110 線維筋痛症	ONO-2020 アルツハイマー型認知症に伴うアジテーション
Phase2	ONO-1110 ハンナ型間質性膀胱炎	ONO-2017 てんかん強直間代発作
Phase2	ONO-1110 うつ病	ONO-2017 てんかん部分発作(小児)
Phase2	ONO-1110 社交不安症	

承認見込み　試験開始　Data Readout

本日のアジェンダ

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POC試験のデータ詳細

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本日のご紹介内容

✓ONO-4578の最新状況

✓ONO-2808の最新状況

ONO-4578-08試験

EP4受容体拮抗薬
HER2陰性 胃がん一次治療

2026 ASCO® ANNUAL MEETING

ONO-4578 combined with nivolumab (NIVO) and chemotherapy (chemo) as first-line (1L) treatment for patients with HER2-negative unresectable advanced or recurrent (adv/rec) gastric/gastroesophageal junction cancer (G/GEJ): A randomized, double-blind, phase 2 trial (ONO-4578-08)

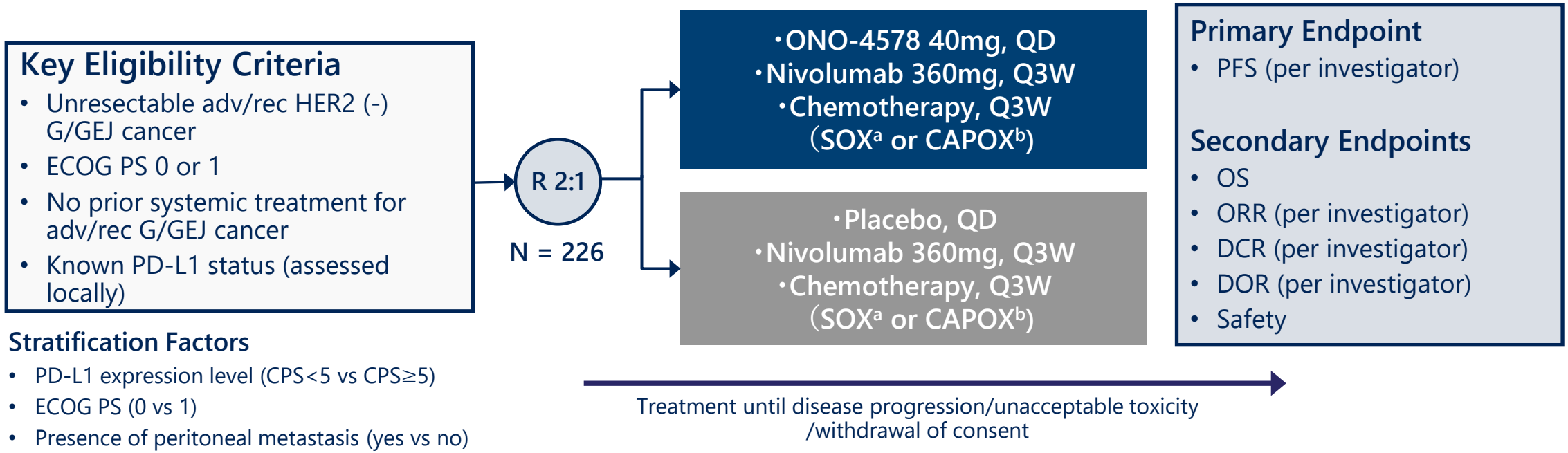
Sung Hee Lim[1*], Izuma Nakayama[2], Min-Hee Ryu[3], Jong Gwang Kim[4], Takeshi Omori[5], Sang Cheul Oh[6], Jin Young Kim[7], Sun Young Rha[8], Keun-Wook Lee[9], Nozomu Machida[10], Sun Jin Sym[11], Yukiya Narita[12], Young-Iee Park[13], Hiroki Hara[14], Hisashi Hosaka[15], Beodeul Kang[16], In-Ho Kim[17], Li-Yuan Bai[18], Kohei Shitara[2], ONO-4578-08 Study Group

[1]Samsung Medical Center, Sungkyunkwan University School of Medicine, Seoul, Republic of Korea; [2]National Cancer Center Hospital East, Kashiwa, Japan; [3]Asan Medical Center, University of Ulsan College of Medicine, Seoul, Republic of Korea; [4]Kyungpook National University, Daegu, Republic of Korea; [5]Osaka International Cancer Institute, Osaka, Japan; [6]Korea University Guro Hospital, Korea University College of Medicine, Seoul, Republic of Korea; [7]Keimyung University Dongsan Hospital, Daegu, Republic of Korea; [8]Yonsei Cancer Center, Yonsei University College of Medicine, Seoul, Republic of Korea; [9]Seoul National University College of Medicine, Seoul National University Bundang Hospital, Seongnam, Republic of Korea; [10]Kanagawa Cancer Center, Yokohama, Japan; [11]Gachon University Gil Medical Center, Incheon, Republic of Korea; [12]Aichi Cancer Center Hospital, Nagoya, Japan; [13]Research Institute and Hospital, National Cancer Center, Goyang, Republic of Korea; [14]Saitama Cancer Center, Saitama, Japan; [15]Gunma Prefectural Cancer Center, Gunma, Japan; [16]CHA Bundang Medical Center, CHA University, Seongnam, Republic of Korea; [17]Seoul St. Mary's Hospital, The Catholic University of Korea, Seoul, Republic of Korea; [18]China Medical University Hospital, Taichung, Taiwan

*Presenting

ONO-4578-08 Trial Design

ONO-4578-08: Asian (Japan/Korea/Taiwan), randomized, double-blinded, Phase 2 trial (NCT06256328)



- **Planned sample size: 210 patients, providing ≥70% power (two-sided α=0.10) to detect a HR of 0.65 (median PFS; 12.3 vs 8.0 months) with 117 events**
- **Patients were randomized from December 2023 to September 2024**
- **All analyses are based on a clinical data cutoff of 14th April 2025, with the median PFS follow-up of 8.5 months**

[a]**SOX therapy:** Oxaliplatin 130 mg/m^2 IV once daily (day1), and S-1 40 mg/m^2/dose orally twice daily (day1-14) , Q6W; [b]**CapeOX therapy:** Oxaliplatin 130 mg/m^2 IV once daily (day1), and Capecitabine 1000 mg/m^2/dose orally twice daily (day1-14), Q3W.

Abbreviations: G/GEJ, gastric/gastroesophageal junction; PFS, progression free survival; OS, overall survival; ORR, objective response rate; DCR, disease control rate; DOR, duration of response; Q3W, every 3 weeks; Q6W, every 6 weeks; SOX, S-1 (tegafur/gimeracil/oteracil)/oxaliplatin; CapeOX, capecitabine/oxaliplatin;

Demographics and Baseline Characteristics

	ONO-4578 group (n = 150)	Placebo group (n = 76)
Age, median (range), years	66.0 (27–84)	67.5 (36–86)
Male sex	113 (75.3)	64 (84.2)
Country		
Japan	54 (36.0)	37 (48.7)
Korea	87 (58.0)	35 (46.1)
Taiwan	9 (6.0)	4 (5.3)
ECOG PS		
0	78 (52.0)	41 (53.9)
1	72 (48.0)	35 (46.1)
Disease status		
Advanced	103 (68.7)	55 (72.4)
Recurrent	47 (31.3)	21 (27.6)
Primary Tumor location[*1]		
GEJ	17 (16.5)	7 (12.7)
Gastric	82 (79.6)	46 (83.6)
Unknown	4 (3.9)	2 (3.6)
Histologic type (Lauren's criteria)		
Intestinal type	72 (48.0)	31 (40.8)
Diffuse type	67 (44.7)	37 (48.7)
Others	11 (7.3)	8 (10.5)
Peritoneal metastasis		
Yes	82 (54.7)	42 (55.3)
No	68 (45.3)	34 (44.7)

	ONO-4578 group (n = 150)	Placebo group (n = 76)
Number of organs with metastases		
≤1	63 (42.0)	29 (38.2)
>1	87 (58.0)	47 (61.8)
PD-L1 expression level (CPS)		
<1	32 (21.3)	17 (22.4)
≥1 and <5	44 (29.3)	21 (27.6)
≥5	73 (48.7)	36 (47.4)
Indeterminate	1 (0.7)	2 (2.6)
Planned chemotherapy regimen		
SOX	89 (59.3)	45 (59.2)
CapeOX	61 (40.7)	31 (40.8)
Claudin 18.2		
Positive	50 (33.3)	28 (36.8)
Negative	96 (64.0)	47 (61.8)
Not Evaluated	4 (2.7)	1 (1.3)
MSI status[*2]		
MSS	32 (21.3)	19 (25.0)
MSI-low	2 (1.3)	0
MSI-high	4 (2.7)	0
Not determined	2 (1.3)	3 (3.9)

*2 The results of patients who locally underwent MSI test

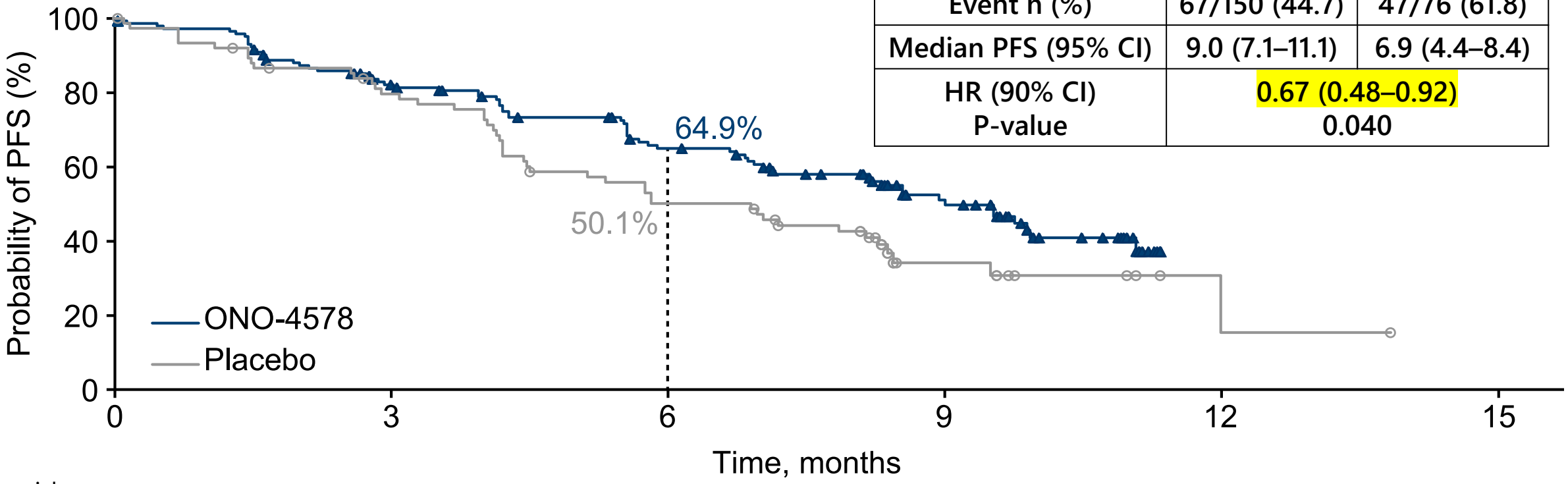
- Baseline characteristics were well balanced across the two groups

*1 Primary tumor location was categorized into three groups: gastroesophageal junction (GEJ; ICD-10 C16.0), gastric (C16.1–C16.4), and unknown ; percentages are based on the number of participants with advanced gastric cancer.

PFS per investigator : Primary Endpoint

median PFS follow-up of 8.5 months

	ONO-4578 group	Placebo group
Event n (%)	67/150 (44.7)	47/76 (61.8)
Median PFS (95% CI)	9.0 (7.1–11.1)	6.9 (4.4–8.4)
HR (90% CI) P-value	0.67 (0.48–0.92) 0.040	



No. at risk

ONO-4578	150	106	77	37	0	0
Placebo	76	57	35	10	1	0

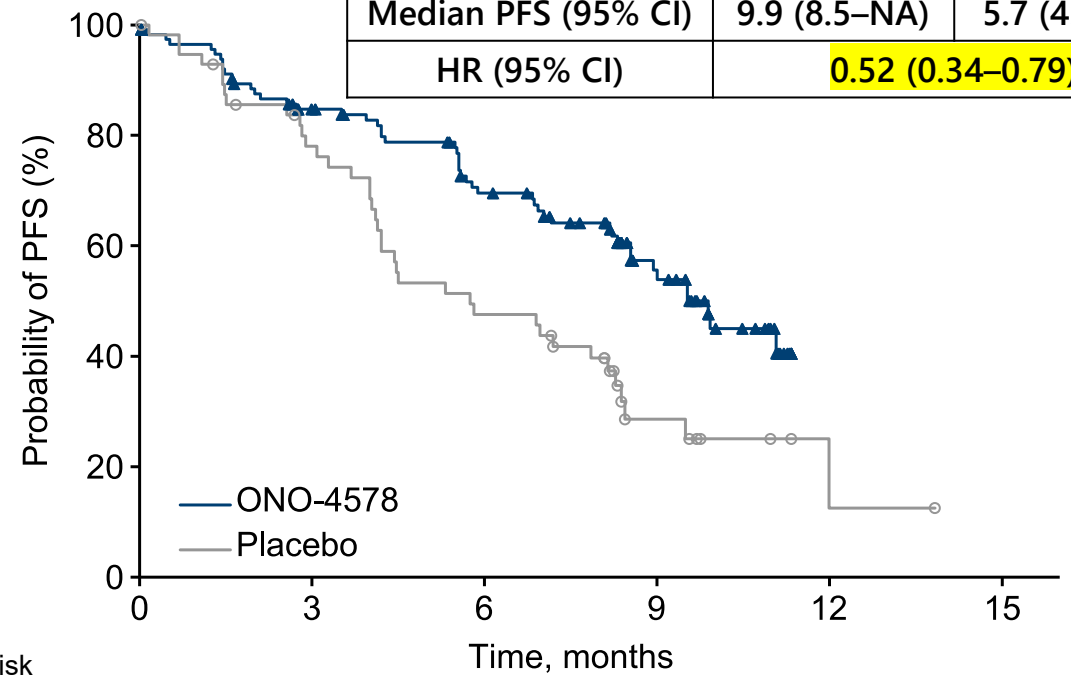
- ONO-4578 group demonstrated a statistically significant improvement in PFS compared with placebo group

PFS by PD-L1 CPS (≥1 vs <1)

median PFS follow-up of 8.5 months

CPS≥1

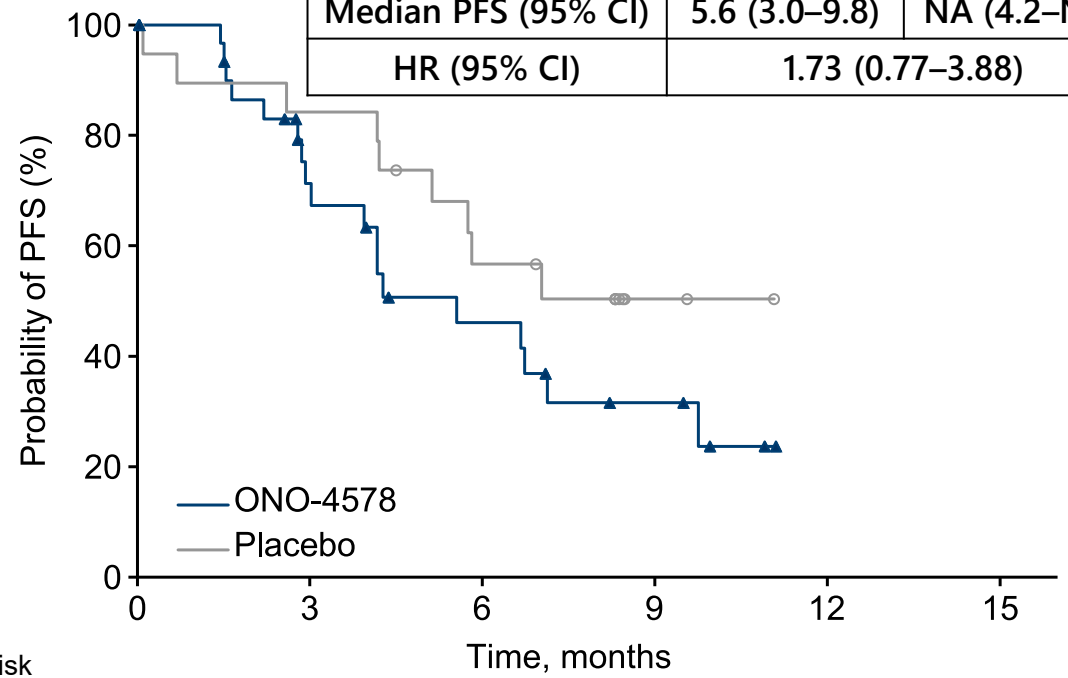
	ONO-4578 group	Placebo group
Event n (%)	49/117 (41.9)	38/57 (66.7)
Median PFS (95% CI)	9.9 (8.5–NA)	5.7 (4.1–8.3)
HR (95% CI)	0.52 (0.34–0.79)	



No. at risk						
ONO-4578	117	88	67	32	0	0
Placebo	57	41	25	8	1	0

CPS<1 or Indeterminate

	ONO-4578 group	Placebo group
Event n (%)	18/33 (54.5)	9/19 (47.4)
Median PFS (95% CI)	5.6 (3.0–9.8)	NA (4.2–NA)
HR (95% CI)	1.73 (0.77–3.88)	

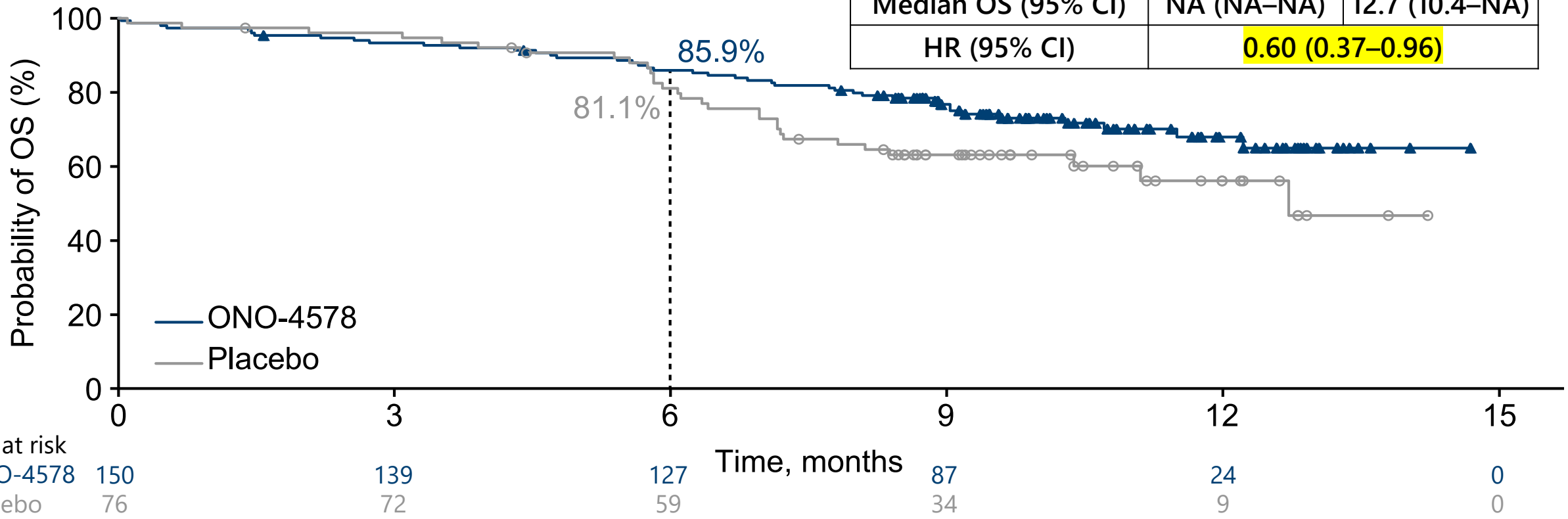


No. at risk						
ONO-4578	33	18	10	5	0	0
Placebo	19	16	10	2	0	0

- **PFS benefit of the ONO-4578 group appeared to be more pronounced in patients with CPS ≥1**

OS : Secondary Endpoint

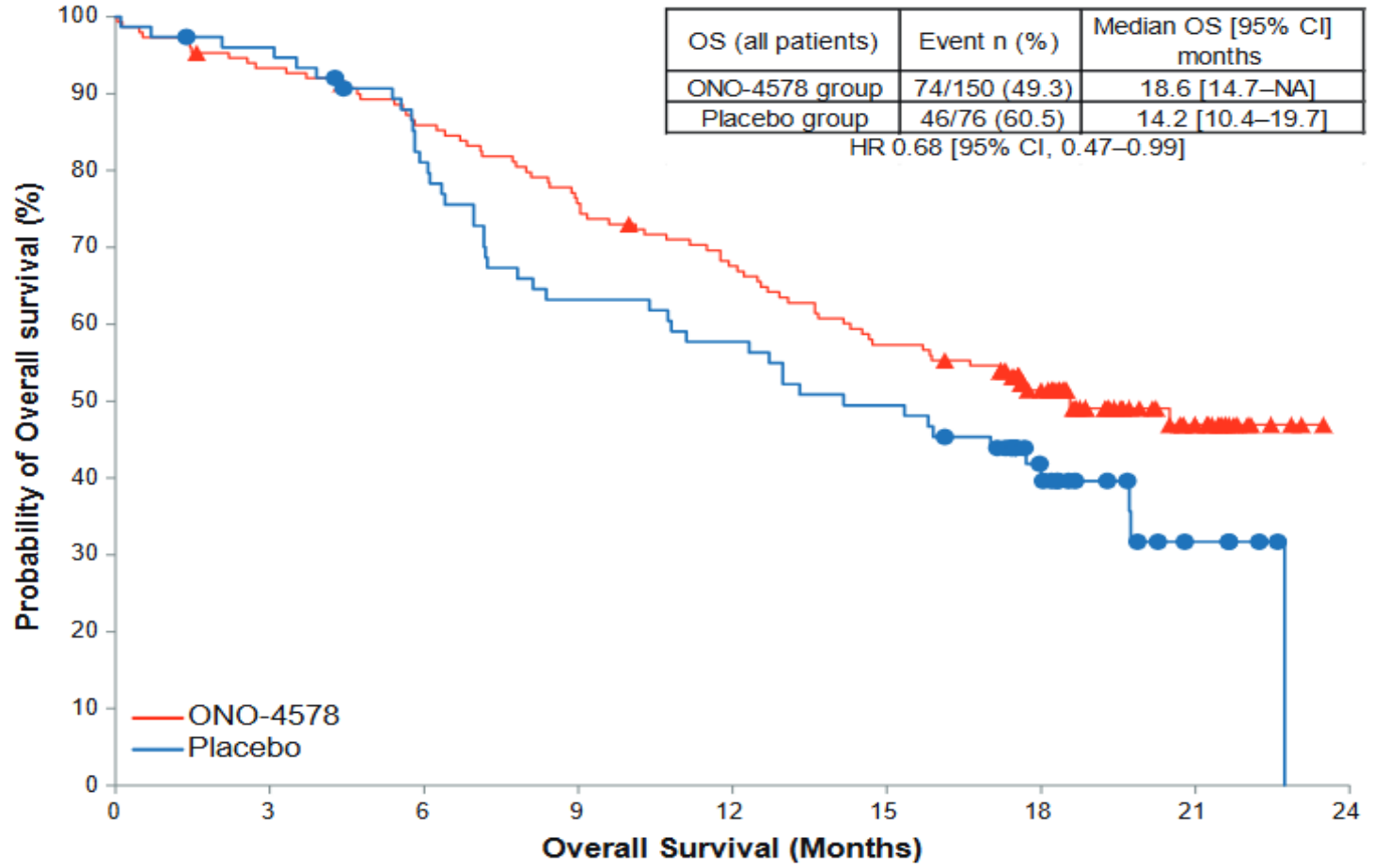
	ONO-4578 group	Placebo group
Event n (%)	42/150 (28.0)	30/76 (39.5)
Median OS (95% CI)	NA (NA–NA)	12.7 (10.4–NA)
HR (95% CI)	0.60 (0.37–0.96)	



- Although OS data are immature (minimum FU: 7.4 months) and should be interpreted with caution, OS favored the ONO-4578 group compared with the placebo group

Post-hoc extended OS

minimum OS follow-up of 16.1 months



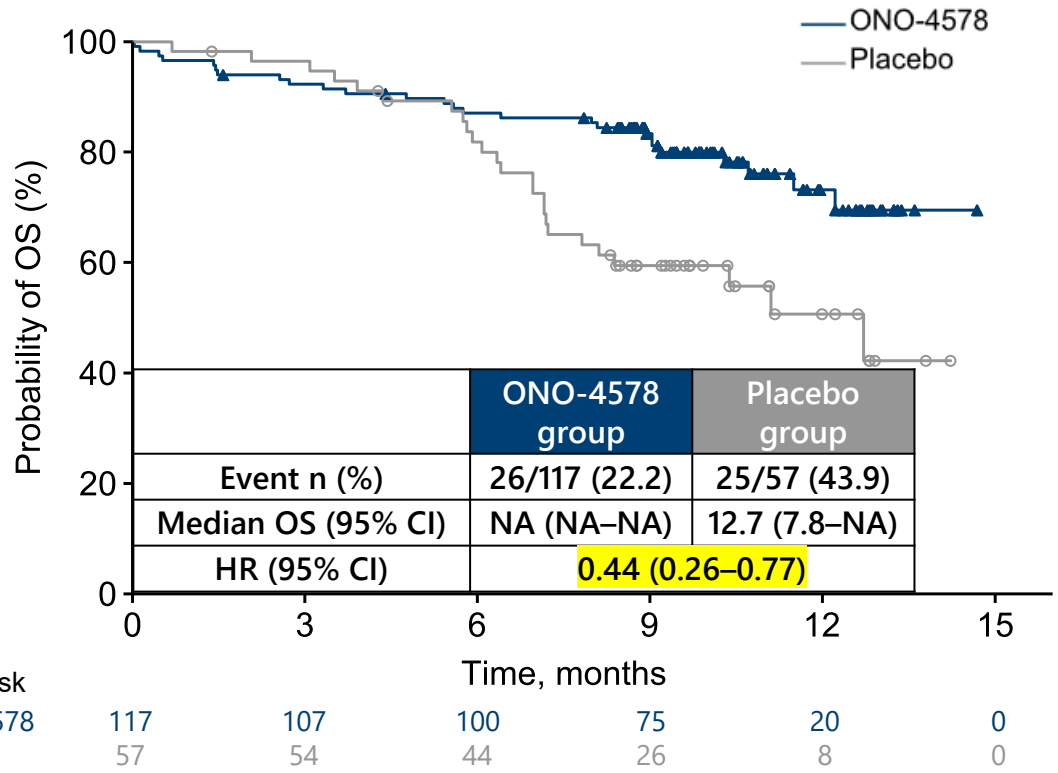
Number of Subjects at Risk

Months	0	3	6	9	12	15	18	21	24
ONO-4578	150	139	127	112	99	84	56	16	0
Placebo	76	72	59	46	42	36	19	5	0

Nakayama I, Ryu M-H, Lim S H, et al. Journal of Clinical Oncology10.1200/JCO-26-01072R1 Epub 2026 June 1.

Overall Survival by PD-L1 CPS (≥1 vs <1)

CPS≥1

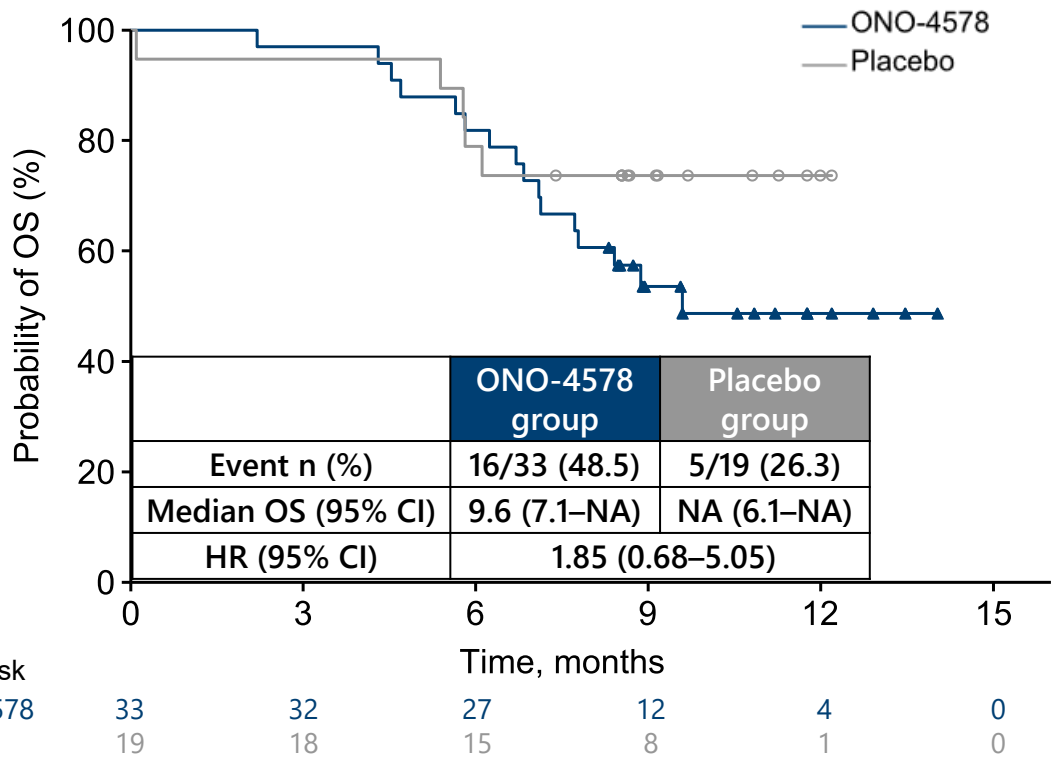


	ONO-4578 group	Placebo group
Event n (%)	26/117 (22.2)	25/57 (43.9)
Median OS (95% CI)	NA (NA–NA)	12.7 (7.8–NA)
HR (95% CI)	0.44 (0.26–0.77)	

No. at risk

ONO-4578	117	107	100	75	20	0
Placebo	57	54	44	26	8	0

CPS<1 or Indeterminate



	ONO-4578 group	Placebo group
Event n (%)	16/33 (48.5)	5/19 (26.3)
Median OS (95% CI)	9.6 (7.1–NA)	NA (6.1–NA)
HR (95% CI)	1.85 (0.68–5.05)	

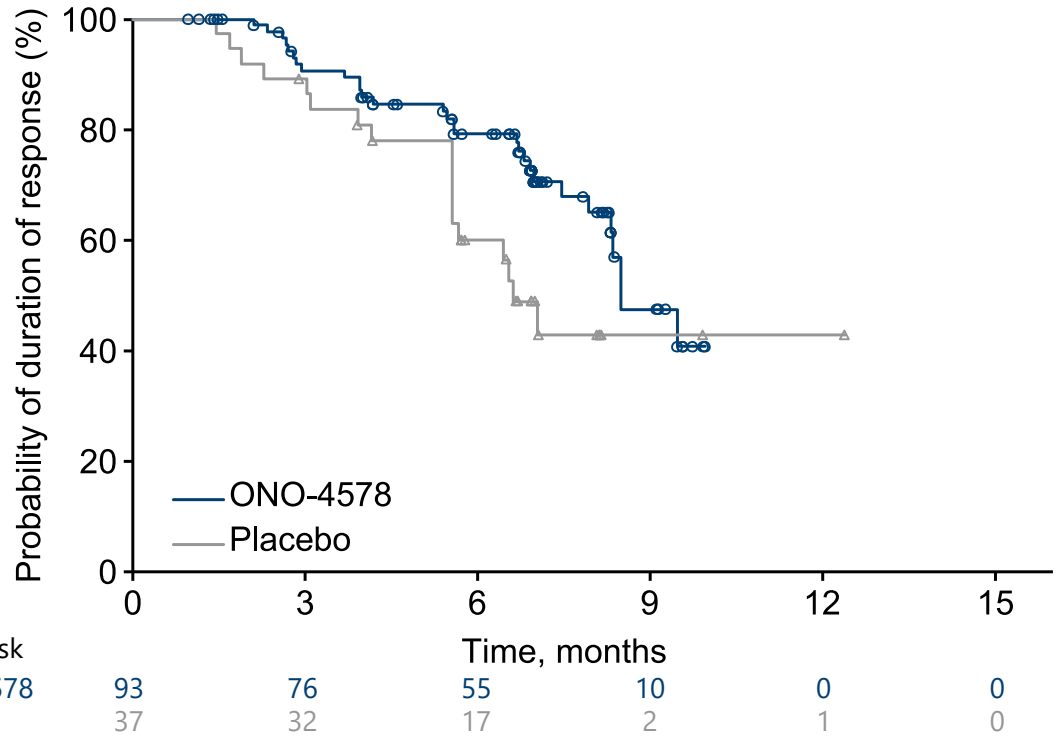
No. at risk

ONO-4578	33	32	27	12	4	0
Placebo	19	18	15	8	1	0

- As with the PFS, the efficacy of the ONO-4578 group was more marked in patients with CPS ≥1

Summary of Anti-tumor Response

	ONO-4578 group (n = 150)	Placebo group (n = 76)
ORR (95% CI), %	62.0 (53.7–69.8)	48.7 (37.0–60.4)
Odds ratio (95% CI)	1.72 (0.98–3.00)	
BOR, n (%)		
CR	4 (2.7)	0
PR	89 (59.3)	37 (48.7)
SD	31 (20.7)	26 (34.2)
PD	12 (8.0)	9 (11.8)
NE	14 (9.3)	4 (5.3)
DOR, median (95% CI), months	8.5 (8.3–N.A.)	6.6 (5.6–N.A.)

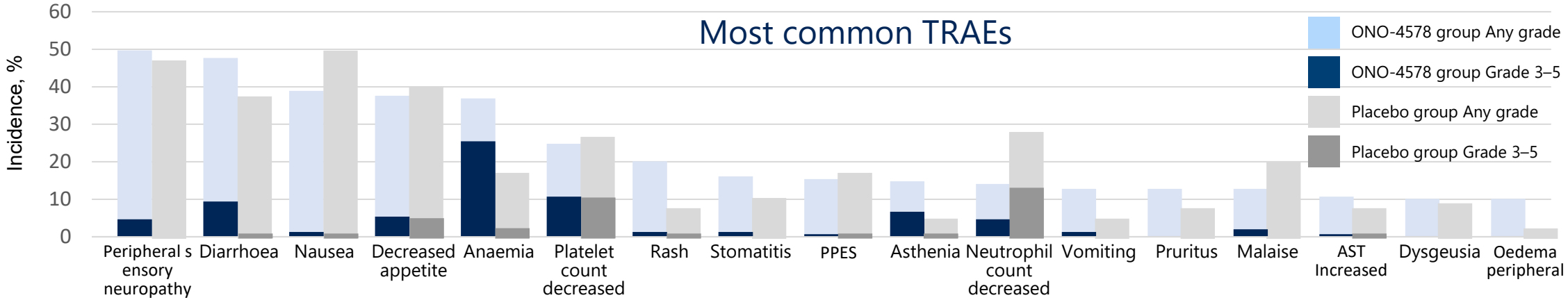


	CPS≥1		CPS<1	
	ONO-4578 group (n = 117)	Placebo group (n = 57)	ONO-4578 group (n = 33)	Placebo group (n = 19)
ORR (95% CI), %	70.9 (61.8–79.0)	50.9 (37.3–64.4)	30.3 (15.6–48.7)	42.1 (20.3–66.5)
Odds ratio (95% CI)	2.36 (1.22–4.54)		0.60 (0.18–1.94)	

- The ONO-4578 group showed higher ORR and longer DOR compared with the placebo groupy

Overall Safety Summary

All treated	ONO-4578 group (n = 149)		Placebo group (n = 75)	
	Any grade	Grade3–5	Any grade	Grade3–5
Any AEs	149 (100.0)	118 (79.2)	75 (100.0)	52 (69.3)
Serious AEs	80 (53.7)	72 (48.3)	32 (42.7)	26 (34.7)
AEs leading to death	12 (8.1)		3 (4.0)	
Any TRAEs	146 (98.0)	89 (59.7)	74 (98.7)	37 (49.3)
Serious TRAEs	51 (34.2)	46 (30.9)	19 (25.3)	16 (21.3)
TRAEs leading to discontinuation of ONO-4578/Placebo	12 (8.1)	7 (4.7)	1 (1.3)	1 (1.3)
TRAEs leading to discontinuation of nivolumab/chemotherapy	58 (38.9)	24 (16.1)	20 (26.7)	6 (8.0)
TRAEs leading to death	4 (2.7)		2 (2.7)	



- TRAEs leading to death were pneumonia klebsiella, febrile neutropenia and hepatitis in the ONO-4578 group, pneumonia interstitial and pneumonia in the placebo group
- The safety profile of ONO-4578 regimen appeared manageable with appropriate supportive care

Abbreviations: PPES, Palmar-plantar erythrodysaesthesia syndrome; AST, Aspartate aminotransferase

ONO-4578-08試験の結果をふまえて

【予後因子】過去の試験との比較（CM649）

✓「65歳以上、Diffuse type、腹膜転移あり」の割合は、CM649と比較して本試験で約10～30%高かった。
✓「PS 1、転移臓器数 2以上」の割合は、CM649と比較して本試験で約10～20%低かった。

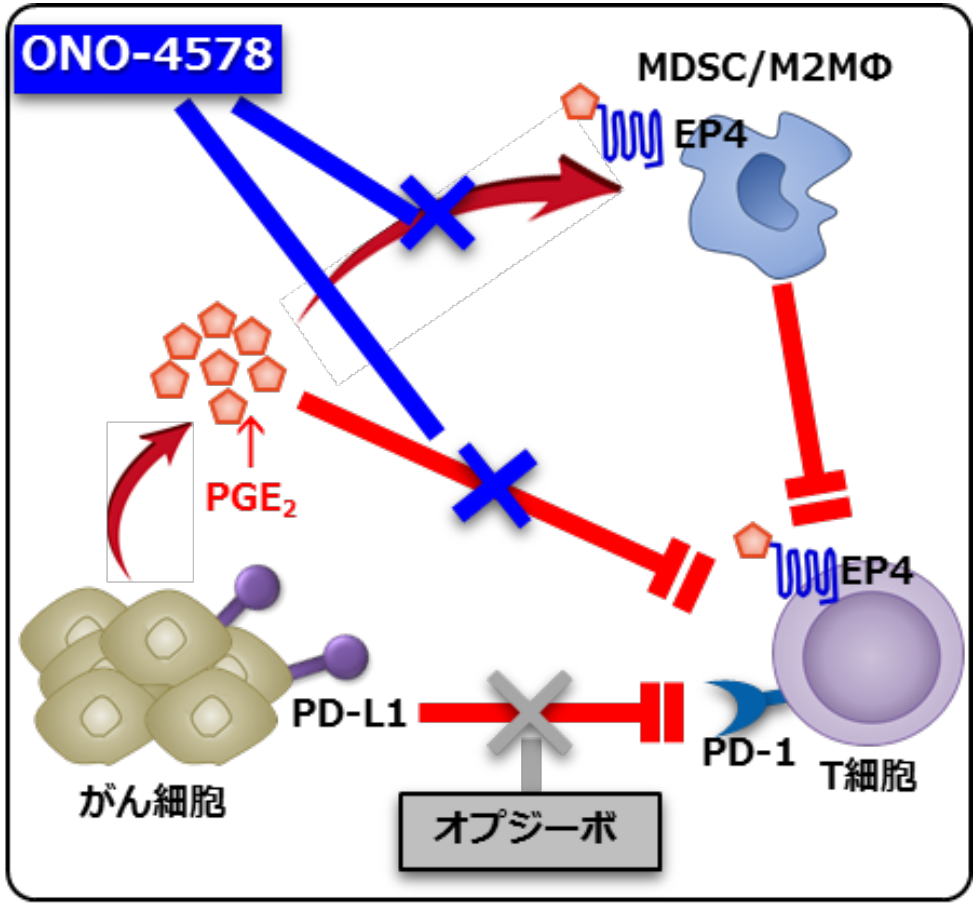
	ONO-4578-08 （CPS測定：施設判定）		Checkmate-649 （CPS測定：中央判定）	
	ONO-4578 group N=150	Placebo group N=76	Nivo + chemo N=789	chemo N=792
Age ≧ 65	80 (53.3%)	49 (64.5%)	316 (40%)	304 (38%)
ECOG PS 1				
0	78 (52.0%)	41 (53.9%)	326 (41%)	336 (42%)
1	72 (48.0%)	35 (46.1%)	462 (59%)	452 (57%)
Histologic type (Lauren's criteria)				
Intestinal type	72 (48.0%)	31 (40.8%)	272 (34%)	267 (34%)
Diffuse type	67 (44.7%)	37 (48.7%)	254 (32%)	273 (34%)
Others	11 (7.3%)	8 (10.5%)	263 (33%)	252 (32%)
Presence of peritoneal metastasis	82 (54.7%)	42 (55.3%)	188 (24%)	188 (24%)
Number of organs with metastases				
≤1	63 (42.0%)	29 (38.2%)	164 (21%)	183 (23%)
≥2	87 (58.0%)	47 (61.8%)	602 (76%)	583 (74%)

ONO-4578の作用機序

- プロスタグランディンE_2（PGE_2）はシクロオキシゲナーゼ-2（COX-2）によりアラキドン酸から生成される。

- COX-2は固形がんで過剰発現している[1)]。PGE_2は、その受容体の一つであるEP4を介して、腫瘍微小環境において骨髄由来抑制細胞（MDSC）やM2マクロファージを誘導し、細胞傷害性T細胞の活性化を抑制することが報告されている[2)]。

- 新規の選択的EP4拮抗剤であるONO-4578は、PGE_2がEP4を介して構築する腫瘍免疫抑制機構を解除することで抗腫瘍効果をもたらすことが期待される。



⇒コンセプト：PD-1抗体薬だけでは克服できない課題を解決

・PD-1抗体が効く患者を増やす＝奏効率を上げる

・PD-1抗体が効かなくなる患者を減らす⇒PFSを延長する

1) Bing L, et al. Cancer Cell Int; 2015:15:106
2) Yukinori T, et al. Front Immunol. 2020;11:324

治癒切除不能な進行・再発胃がん（HER2陰性）　一次治療

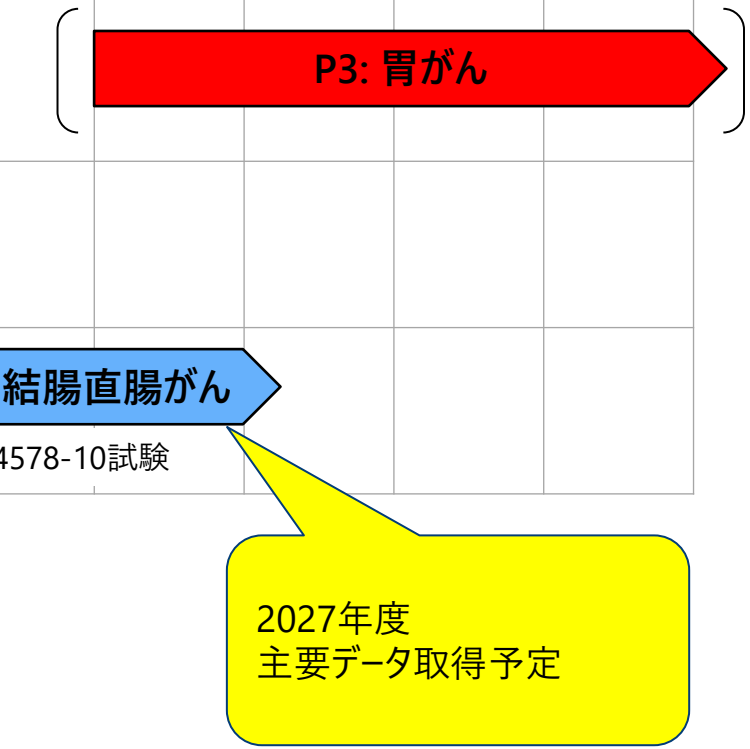
	日本	米国	欧州
標準治療	**ニボルマブ** ＋フッ化ピリミジン＋プラチナ系薬剤	[CPS≧1] **ニボルマブ／ペムブロリズマブ** ＋フッ化ピリミジン＋プラチナ系薬剤	[CPS≧5] **ニボルマブ／ペムブロリズマブ** ＋フッ化ピリミジン＋プラチナ系薬剤
	ペムブロリズマブ ＋フッ化ピリミジン＋プラチナ系薬剤	[TAP≧1%] **チスレリズマブ** ＋フッ化ピリミジン＋プラチナ系薬剤	[TAP≧5%] **チスレリズマブ** ＋フッ化ピリミジン＋プラチナ系薬剤
		[CPS<5又はCPS<1]　フッ化ピリミジン＋プラチナ系薬剤	
	ゾルベツキシマブ＋フッ化ピリミジン＋プラチナ系薬剤[Claudin18.2陽性]		

	日本	米国	欧州
①HER2陰性（77～88%）#	22,700名	8,900名	22,200名
①×CPS≧1（78%）#	17,700名	6,900名	17,300名
①×CLDN陽性（38%）#	8,600名	3,400名	8,400名
①×ダブルポジティブ（25.7%）*	5,900名	2,300名	5,700名

#James Yu and Rutika Mehta. Journal of the National Comprehensive Cancer Network; 2025:23:5
* Nakayama I, Ryu M-H, Lim S H, et al. Journal of Clinical Oncology10.1200/JCO-26-01072R1 Epub 2026 June 1.

ONO-4578 開発状況

適応症等	開発相	進捗	実施国	試験番号
一次治療 胃がん*	PIII	試験開始準備中		
一次治療 胃がん*	P II	2025年度主要 データ取得済み	日韓台	NCT06256328
一次治療 結腸・直腸がん*	P II	2027年度主要 データ取得	日米欧など	NCT06948448



*オプジーボ及び標準治療との併用

2024 ESMO GASTROINTESTINAL CANCERS
Annual Congress
MUNICH GERMANY
26-29 JUNE 2024

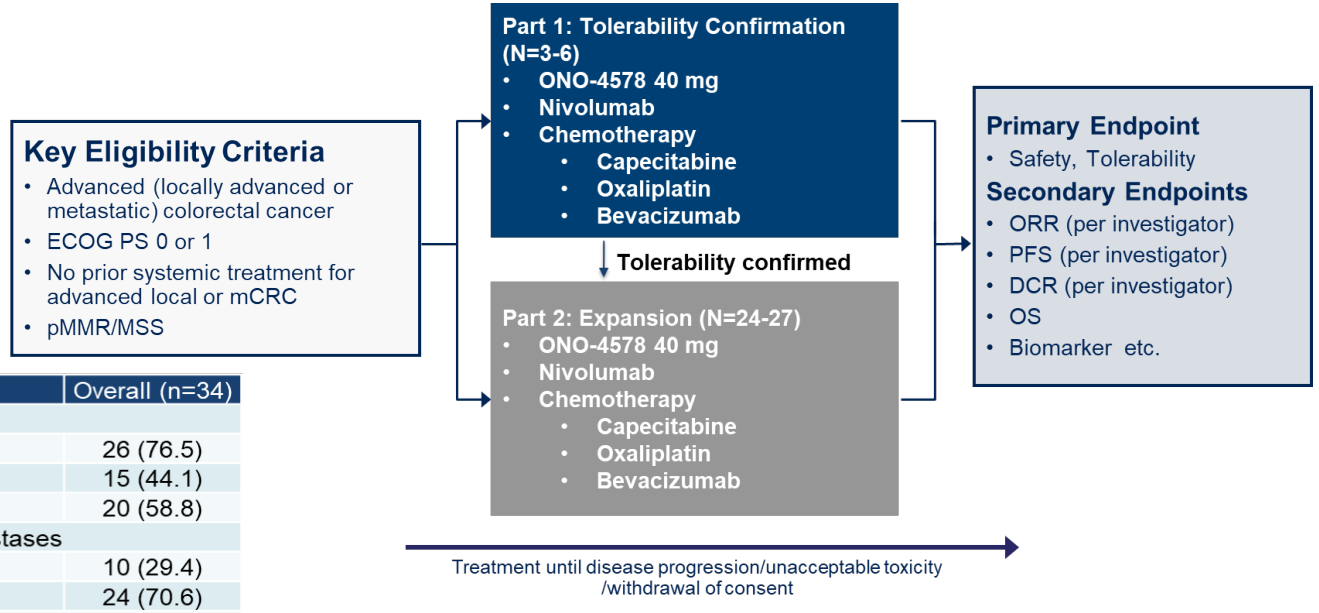
Safety, Efficacy, And Biomarkers Of ONO-4578, An EP4 Antagonist, In Combination With Nivolumab And Chemotherapy In Treatment-naive And Proficient Mismatch Repair (pMMR)/Microsatellite Stable (MSS) Metastatic Colorectal Cancer (mCRC)

Yoshinori Kagawa1, Takeshi Kato2, Manabu Shiozawa3, Kensei Yamaguchi4, Masahiro Goto5, Hisateru Yasui6, Tetsuya Hamaguchi7, Hiroki Hara8, Takayuki Yoshino9

1Department of Gastrointestinal Surgery, Osaka General Medical Center, Osaka, Japan, 2Colorectal Surgery Dept., NHO Osaka National Hospital, Osaka, Japan, 3Gastrointestinal Surgery, Kanagawa Cancer Center, Yokohama, Japan, 4Gastroenterological Chemotherapy Dept., The Cancer Institute Hospital of JFCR, Koto-ku, Japan, 5Cancer Chemotherapy Center, Osaka Medical and Pharmaceutical University Hospital Clinical Research Center, Takatsuki, Japan, 6Medical Oncology Department, Kobe City Medical Center General Hospital, Kobe, Japan, 7Gastroenterological Oncology Dept., Saitama Medical University International Medical Center, Hidaka, Japan, 8Gastroenterology, Saitama Cancer Center, Ina, Japan, 9Department of Gastroenterology and Gastrointestinal Oncology, National Cancer Center Hospital East, Kashiwa, Japan

Study Design & Patient Characteristics

- The ONO-4578-02 study (NCT06547385) was an open-labelled, phase 1 study, conducted at 9 sites in Japan
- The minimum follow-up was 15.0 months at data cutoff (July 21, 2023)
- Patients with high frequency microsatellite instability (MSI-H) or mismatch repair mechanism deficiency (dMMR) were excluded per protocol



Characteristics	Overall (n=34)
Age (years)	
Median	66.0
Min-Max	40-80
Sex	
Female	17 (50.0)
Male	17 (50.0)
ECOG PS at baseline	
0	30 (88.2)
1	4 (11.8)
Initial or recurrent	
Initial	31 (91.2)
Recurrent	3 (8.8)
Organ location of initial disease	
Left	26 (76.5)
Rectum	16 (47.1)
Sigmoid colon	9 (26.5)
Rectosigmoid Junction	1 (2.9)
Right	8 (23.5)
Cecum	3 (8.8)
Ascending colon	2 (5.9)
Transverse colon	3 (8.8)
Disease stage	
IV	31 (91.2)
Missing	3 (8.8)

Characteristics	Overall (n=34)
Organ location of metastasis	
Liver	26 (76.5)
Lung	15 (44.1)
Lymph node	20 (58.8)
Number of organs showing metastases	
≤1	10 (29.4)
≥2	24 (70.6)
Prior treatment	
Colorectal cancer specific surgeries	8 (23.5)
Radiotherapies	0
Colorectal cancer specific medications	2 (5.9)
BRAF mutation status	
V600E	3 (8.8)
Wild type/No mutation	31 (91.2)
RAS mutation status[a]	
Mutated	21 (61.8)
Wild type	13 (38.2)
PD-L1 CPS	
<1	14 (41.2)
≥1	17 (50.0)
Indeterminate/Unknown	1 (2.9)
Missing	2 (5.9)
TMB (Muts/Mb)	
<10	29 (85.3)
≥10	0
Missing	5 (14.7)

Data presented as n (%) unless specified otherwise; [a]*RAS* mutation present means *KRAS* or *NRAS* mutation present; CPS, combined positive score; ECOG PS, eastern cooperative oncology group performance status; Min, minimum; Max, maximum; Muts/Mb, mutations per megabase; PD-L1, programmed cell death-ligand 1; TMB, tumour mutation burden

Results: Efficacy（全体集団）

	n (%)	[95% CI]
Objective response rate[a]	25 (73.5)	[55.6, 87.1]
Disease control rate[a]	31 (91.2)	[76.3, 98.1]
Best overall response[a]		
Complete Response	0	[0.0, 10.3]
Partial Response	25 (73.5)	[55.6, 87.1]
Stable Disease	6 (17.6)	[6.8, 34.5]
Progressive Disease	2 (5.9)	-
Not Evaluable	1 (2.9)	-
Progression free survival in months[b]		
Median [95% CI]	12.3	[7.0, 17.1]
Progression free survival rate[b]		
At 6 months (%) [95% CI]	84.7	[67.1, 93.4]
At 12 months (%) [95% CI]	50.3	[31.2, 66.7]

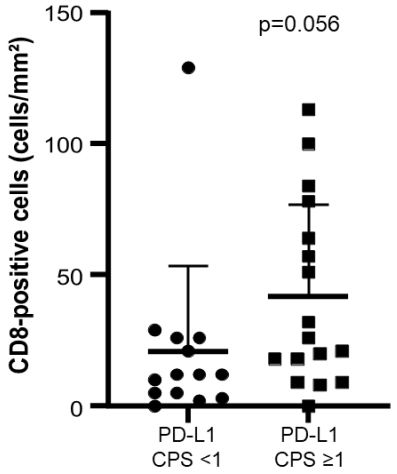
[a]by Clopper-Pearson method ; [b]by Kaplan-Meier method; CI, confidence interval

Results: Efficacy & BM Analysis（CPS陽性/陰性）

	PD-L1 CPS ≥1 (n=17)	PD-L1 CPS <1 (n=14)
Objective response rate, n (%) [95% CI][a]	15 (88.2) [63.6, 98.5]	9 (64.3) [35.1, 87.2]
Progression free survival in months[b]		
Median [95% CI]	12.3 [6.9, NA]	7.4 [6.9, NA]
Progression free survival rate		
At 6 months (%) [95% CI]	88.2 [60.6, 96.9]	85.7 [53.9, 96.2]
At 12 months (%) [95% CI]	57.4 [30.6, 77.0]	46.8 [19.6, 70.2]

[a]by Clopper-Pearson method; [b]by Kaplan-Meier method; CI, confidence interval, CPS, combined positive score; NA, not available; PD-L1, programmed cell death-ligand 1

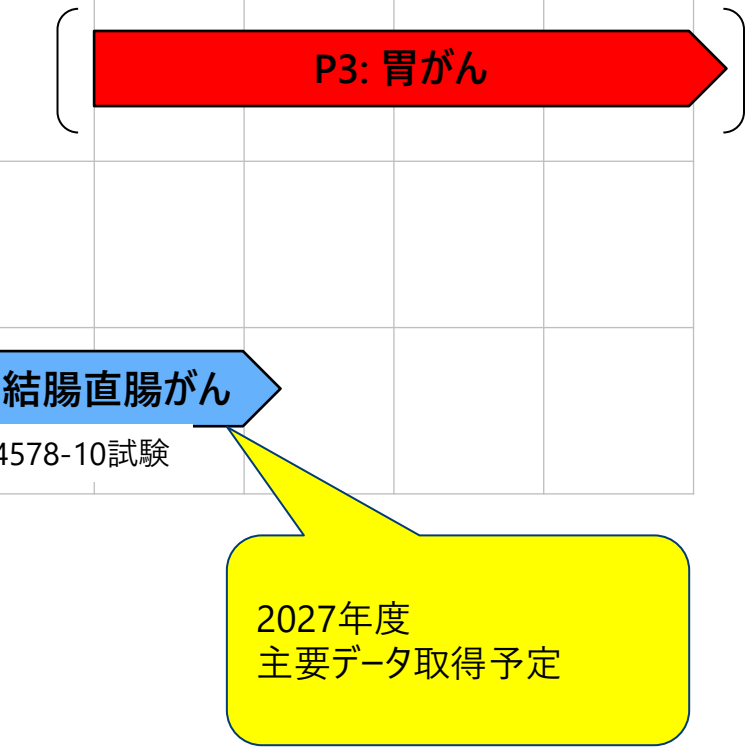
CD8⁺ Immunohistochemistry in PD-L1 CPS subgroups in Tumor Biopsies at Baseline



CPS, combined positive score; PD-L1, programmed cell death-ligand 1

ONO-4578 開発状況（第 II 相試験以降）

適応症等	開発相	進捗	実施国	試験番号
一次治療 胃がん*	PIII	試験開始準備中		
一次治療 胃がん*	P II	2025年度主要 データ取得済み	日韓台	NCT06256328
一次治療 結腸・直腸がん*	P II	2027年度主要 データ取得	日米欧など	NCT06948448



*オプジーボ及び標準治療との併用

ONO-2808-03試験

S1P5受容体選択的作動薬

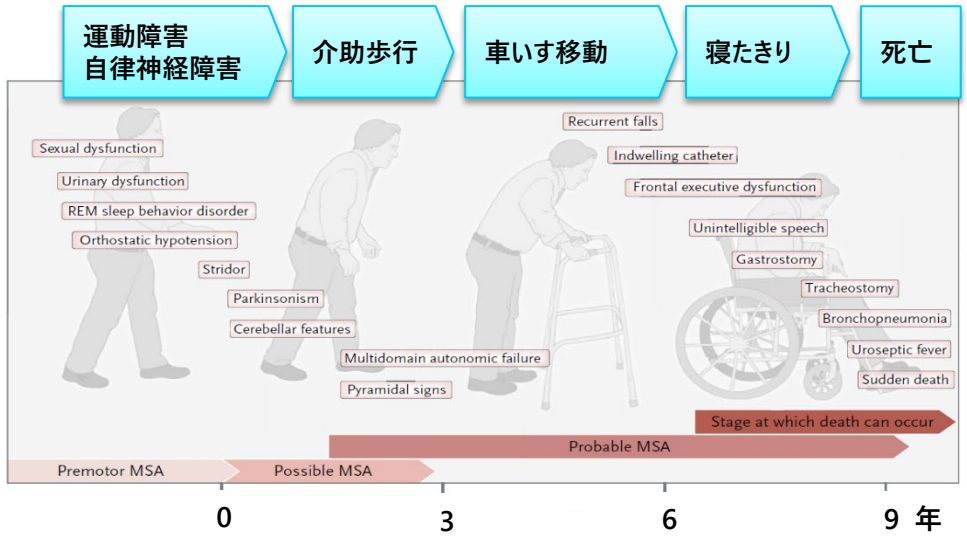
多系統萎縮症（MSA；Multiple System Atrophy）

ONO-2808と多系統萎縮症

化合物の概要

化合物名	ONO-2808
起源会社	小野薬品工業株式会社
作用機序	S1P5受容体作動作用
剤型	経口剤
適応症	多系統萎縮症
開発状況	第Ⅱ相試験（米国、日本）

多系統萎縮症
（MSA：Multiple System Atrophy）

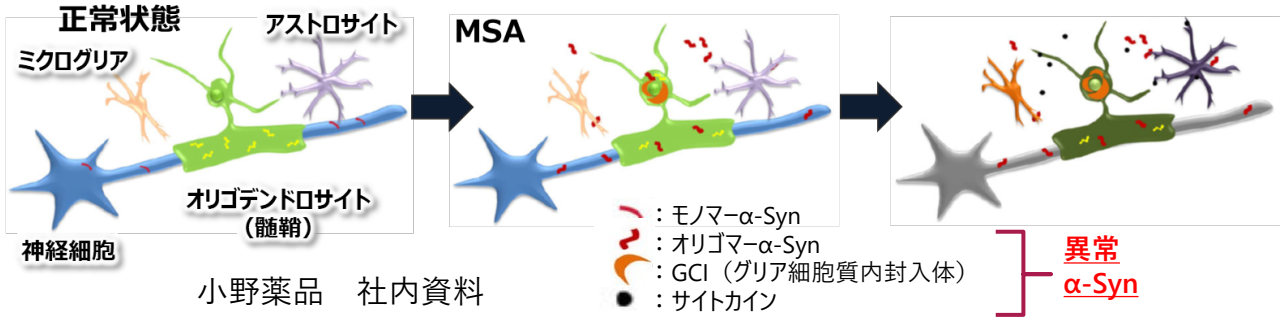


Gregor K. Wenning, N Engl J Med 2015; 372(3)

<特　徴>

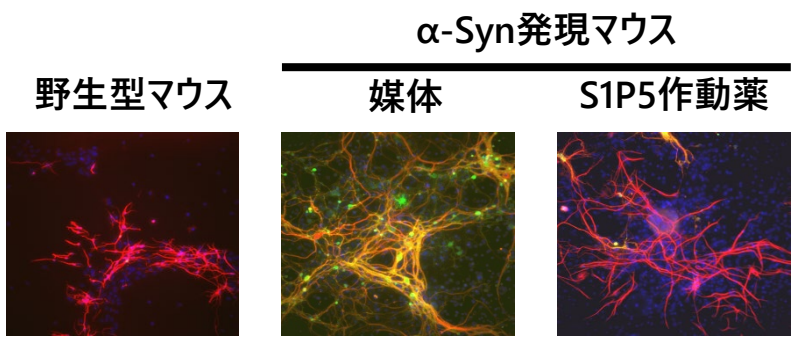
- 小脳などが萎縮する進行性神経変性疾患
- 平均発症年齢：55～60歳
- 重篤かつ進行が早い
- 対症療法しかなく、効果も乏しい
- 患者数（2031年推定）
  米国：1.6万人、欧州5＊：1.6万人、日本：1.2万人

＊欧州5：フランス、ドイツ、イタリア、スペイン、イギリス



小野薬品　社内資料

オリゴデンドロサイト特異的ヒト型α-Syn発現マウス由来の全脳初代培養細胞



青：核
緑：α-Syn
赤：神経細胞

S1P5作動薬は神経軸索のα-Syn蓄積を抑制

Poster: LBP37.09

Safety, Tolerability, and Preliminary Efficacy of ONO-2808, a Sphingosine-1-Phosphate Receptor 5 Agonist, in Multiple System Atrophy

Anne-Marie Wills[1], U Shivraj Sohur[2], Atsushi Takeda[3], Susan Perlman[4], Tomoko Oeda[5], Carlos Singer[6], Daniel D Truong[7], Pravin Khemani[8], Praveen Dayalu[9], Pinky Agarwal[10], Barbara Kelly Changizi[11], Patricio Millar Vernetti[12], Keiko Toyooka[13], Nicole Owens[2], Okan U Elci[2], Ryunosuke Higashi[14], Akihisa Nishimura[14], Rajesh Pahwa[15], Stuart H Isaacson[16], Peter A LeWitt[17], Nikolaus R McFarland[18]

[1]Harvard Medical School, Massachusetts General Hospital, Boston, MA, USA; [2]Ono Pharma USA Inc, Wellesley, MA, USA; [3]Kendall-Hidetsugu Hospital, Sendai, Japan; [4]David Geffen School of Medicine, University of California Los Angeles, Los Angeles, CA, USA; [5]National Hospital Organization, Utano National Hospital, Kyoto, Japan; [6]Miller School of Medicine, University of Miami, Miami, FL, USA; [7]The Parkinson's and Movement Disorder Institute, Fountain Valley, CA, USA; [8]Stanford University, Palo Alto, CA, USA; [9]Cedars-Sinai Medical Center, Los Angeles, CA, USA; [10]Rush University Medical Center, Chicago, IL, USA; [11]Department of Neurology, Dysautonomia Center, Langone Medical Center, New York University Grossman School of Medicine, New York, NY, USA; [12]Department of Neurology, National Hospital Organization Osaka Toneyama Medical Center, Toyonaka, Japan; [13]ONO Pharmaceutical Co., Ltd., Osaka, Japan; [14]University of Kansas Medical Center, Kansas City, KS, USA; [15]Parkinson's Disease and Movement Disorders Center of Boca Raton, Boca Raton, FL, USA; [16]Department of Neurology and Pharmacology, Wayne State University School of Medicine, Detroit, MI, USA; [17]Norman Fixel Institute for Neurological Diseases, University of Florida, Gainesville, FL, USA

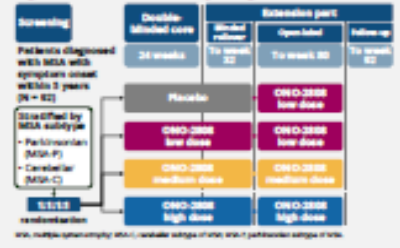
Introduction

- Multiple system atrophy (MSA) is a rare neurodegenerative disorder characterized by glial α-synuclein cytoplasmic inclusions[1]
- The sphingosine-1-phosphate receptor 5 (S1PR5) promotes mature oligodendrocyte survival and contributes to the regulation of myelination, which are key pathologic processes in MSA[2]
- Patients with MSA experience rapid progressive degeneration across motor, non-motor, and autonomic pathways leading to substantial reductions in quality of life and eventual death[3,4]
- MSA can be classified into 2 subtypes based on the predominant phenotype: MSA-P for parkinsonian features associated with striatonigral degeneration or MSA-C for cerebellar features associated with olivopontocerebellar atrophy[3,4]
- There are no approved treatments for MSA, and existing therapies are limited to symptomatic relief rather than addressing underlying pathogenic mechanisms, highlighting an urgent, unmet need for novel therapeutic strategies
- ONO-2808 is an investigational, first-in-class, oral, S1PR5 agonist that preclinically promoted remyelination and reduced α-synuclein accumulation
- Here, we report the safety and exploratory efficacy of ONO-2808 in patients with MSA from the phase 2 trial (NCT05923866)

Methods

- ONO-2808-03 is an ongoing, double-blind, placebo-controlled phase 2 trial in patients with MSA diagnosed per Movement Disorder Society Criteria with symptom onset within 5 years (**Figure 1**)
- The trial is composed of 2 parts, including the double-blinded core part (to week 24; the last patient reached week 24 on September 15, 2025) and extension part, comprising blinded rollover (to week 32), open-label treatment (to week 80), and follow-up (to week 92)
  - Patients who continued in the optional blinded rollover and open-label extension provided additional informed consent, which potentially influenced patient-reported outcomes
- The primary endpoint at week 24 included the incidence of treatment-emergent adverse events (TEAEs) and transaminase elevations assessed by an independent data monitoring committee
- Exploratory efficacy endpoints included here are mean change from baseline (CFB) in a modified Unified Multiple System Atrophy Rating Scale (mUMSARS) score and percent CFB in brain regions of interest as assessed via volumetric magnetic resonance imaging (vMRI)
  - The mUMSARS was an adjusted sum of 9 items (6 items from UMSARS part 1 and 3 items from UMSARS part 2) with scores ranging from 0 to 27 (lower scores indicate less impairment)

Figure 1. ONO-2808-03 study design



Results

- A total of 92 patients with MSA were enrolled across investigational sites in the US and Japan; there were no major imbalances in baseline characteristics between treatment arms (**Table 1**)
- Overall, 76% (70/92) of patients completed treatment in the double-blinded core part, and 57% (52/92) remained on treatment at data cutoff (February 24, 2026)

Table 1. Baseline demographics and clinical characteristics

Characteristic	Placebo (n = 23)	ONO-2808 low (n = 23)	ONO-2808 medium (n = 23)	ONO-2808 high (n = 23)	ONO-2808 total (n = 69)
Age, years					
Mean (SD)	59.6 (6.7)	63.2 (7.8)	60.6 (4.5)	60.4 (7.7)	62.0 (6.9)
Median (range)	58 (51–76)	63 (46–80)	61 (54–74)	60 (49–73)	62 (46–80)
Sex, n (%)					
Male	15 (65)	9 (39)	14 (61)	13 (57)	36 (52)
Female	8 (35)	14 (61)	9 (39)	10 (43)	33 (48)
Race, n (%)					
White	15 (65)	18 (78)	16 (57)	15 (65)	46 (67)
Asian	7 (30)	3 (13)	9 (39)	6 (26)	18 (26)
Other[a]	1 (4)	2 (9)	1 (4)	2 (9)	5 (7)
Years since diagnosis, n (%)					
0 to <1	8 (35)	6 (26)	10 (43)	8 (35)	24 (35)
1 to <2	10 (43)	10 (43)	6 (26)	9 (39)	25 (36)
2 to <3	3 (13)	4 (17)	3 (13)	2 (9)	9 (13)
3 to <4	2 (9)	3 (9)	3 (13)	3 (13)	8 (12)
4 to <5	0	1 (4)	1 (4)	1 (4)	3 (4)
MSA subtype, n (%)					
MSA-P	13 (57)	11 (48)	12 (52)	12 (52)	35 (51)
MSA-C	10 (43)	12 (52)	11 (48)	11 (48)	34 (49)

[a]Includes Black/African American and American Indian/Alaska Native.
MSA, multiple system atrophy; MSA-C, cerebellar subtype of MSA; MSA-P, parkinsonian subtype of MSA; SD, standard deviation.

Safety

- During the double-blinded core part, most patients experienced ≥1 any-grade TEAE (93% [64/69] across all ONO-2808 doses and 91% [21/23] in the placebo group)
- Treatment-related TEAEs led to treatment interruption in 4% (3/69) of patients across all ONO-2808 doses and 4% (1/23) in the placebo group, and discontinuation in 13% (9/69) and 4% (1/23), respectively
- The most frequent TEAEs with ONO-2808 were urinary tract infection, headache, constipation, fall, and nasopharyngitis (**Table 2**)
- Transaminase elevations occurred in 12% (8/69) of patients across ONO-2808 dose levels; all were reversible, and none met Hy's law criteria (**Table 3**)
- As of February 24, 2026, there were no new safety signals in the open-label extension period

Table 2. TEAEs in ≥10% of patients in any treatment group during the double-blinded core part

Preferred term, n (%)	Placebo (n = 23)	ONO-2808 low (n = 23)	ONO-2808 medium (n = 23)	ONO-2808 high (n = 23)	ONO-2808 total (n = 69)
Urinary tract infection	8 (35)	9 (39)	2 (9)	4 (17)	15 (22)
Headache	0	3 (13)	2 (9)	6 (26)	11 (16)
Constipation	0	1 (4)	0	4 (17)	5 (7)
Fall	3 (13)	3 (13)	2 (9)	0	5 (7)
Nasopharyngitis	1 (4)	0	2 (9)	3 (13)	5 (7)
Contusion	4 (17)	2 (9)	1 (4)	1 (4)	4 (6)
Fatigue	3 (13)	1 (4)	1 (4)	2 (9)	4 (6)
Arthralgia	4 (17)	1 (4)	1 (4)	1 (4)	3 (4)
Dizziness	1 (4)	0	0	3 (13)	3 (4)
Diarrhea	3 (13)	1 (4)	1 (4)	0	2 (3)
Skin abrasion	3 (13)	0	0	0	0

Data from the double-blind core part, which included results with 24 weeks of follow-up.
TEAE, treatment-emergent adverse event.

Table 3. Transaminase elevations adjudicated by the IDMC at week 24

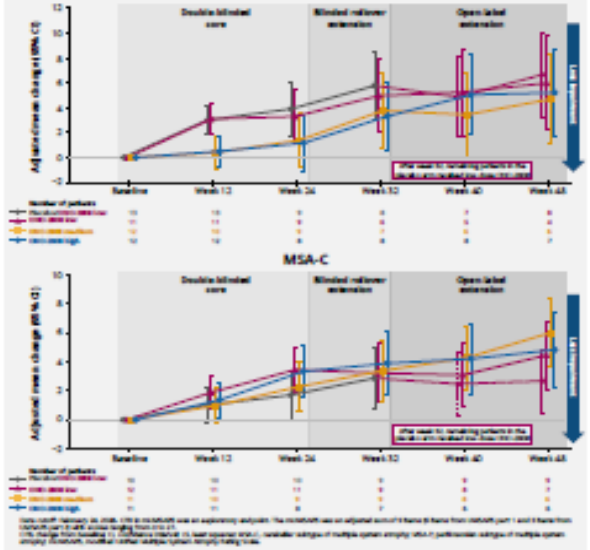
Category, n (%)	Placebo (n = 23)	ONO-2808 low (n = 23)	ONO-2808 medium (n = 23)	ONO-2808 high (n = 23)	ONO-2808 total (n = 69)
Any transaminase elevation confirmed by IDMC	0	1 (4)	3 (13)	4 (17)	8 (12)
Any treatment-related transaminase elevation by highest severity					
Mild	0	0	1 (4)	2 (9)	3 (4)
Moderate	0	0	1 (4)	2 (9)	3 (4)
Severe	0	1 (4)	1 (4)	0	2 (3)
Any treatment-related transaminase elevation leading to withdrawal of treatment	0	1 (4)	3 (13)	3 (13)	7 (10)
Any serious transaminase elevations	0	1 (4)	0	0	1 (1)
Treatment-related	0	1 (4)	0	0	1 (1)

Data from the double-blind core part, which included results with 24 weeks of follow-up.
IDMC, independent data monitoring committee.

Efficacy

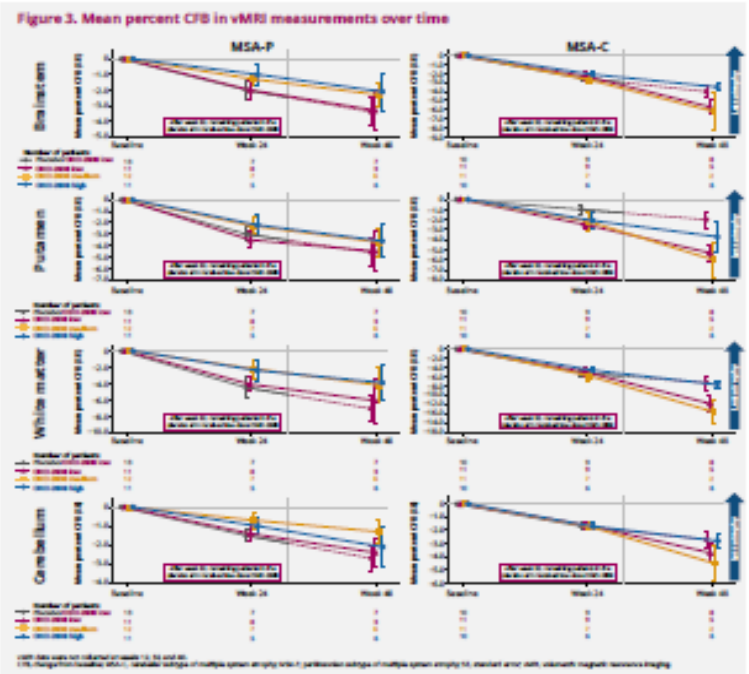
- The mean CFB (95% confidence interval [CI]) mUMSARS at week 24 showed attenuation of scores in patients with MSA-P in the ONO-2808 medium- (1.39 [−0.85 to 3.64]) and high-dose (1.16 [−1.1 to 3.41]) groups compared with placebo (3.90 [1.76 to 6.04]; **Figure 2**)
  - Attenuation of mUMSARS scores in patients with MSA-P were sustained up to week 48 in the ONO-2808 groups compared with patients randomized to placebo

Figure 2. LS Mean CFB in mUMSARS over time



- A dose-dependent response was observed in slowing of brain atrophy as assessed by vMRI in the brainstem, putamen, white matter, and cerebellum in patients with MSA-P treated with ONO-2808 vs those randomized to placebo placebo (**Figure 3**)

Figure 3. Mean percent CFB in vMRI measurements over time



CONCLUSIONS

- ONO-2808 had an acceptable safety profile in patients with MSA, and no new safety signals were observed with continued follow-up as of February 2026
  - All transaminase elevations across ONO-2808 doses were reversible, and none met Hy's law criteria
- In exploratory efficacy analyses up to week 48, ONO-2808 demonstrated a potential signal of efficacy as assessed by mUMSARS in patients with MSA-P
- ONO-2808 demonstrated slowing of brain atrophy over time vs placebo by vMRI in patients with MSA-P. The manageable safety profile and preliminary signs of efficacy support the continued investigation of ONO-2808 as a potential treatment to slow disease progression in patients with MSA

PRESENTED AT THE 2026 WORLD PARKINSON CONGRESS
MAY 24–27, 2026; PHOENIX, AZ, USA

CORRESPONDING AUTHOR
Anne-Marie Wills
awills@mgh.harvard.edu

ACKNOWLEDGMENTS
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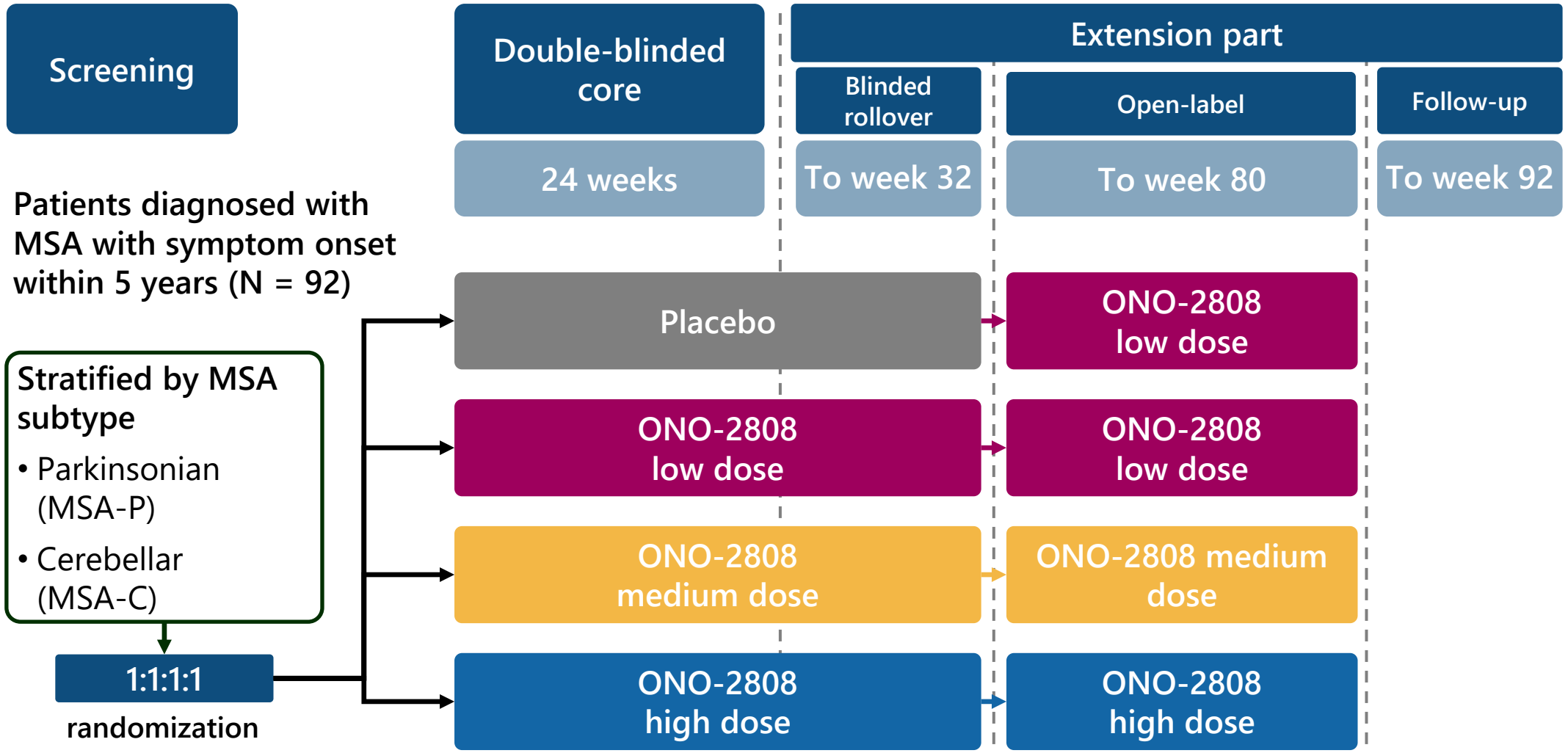
1. Gilman S, et al. Neurology. 2008;71(9):670-676.
2. Mitew S, et al. J Neurosci. 2013;33(12):4935-4946.
3. Wenning GK, et al. Mov Disord. 2022;37(6):1131-1148.
4. Stefanova N, et al. Trends Neurosci. 2005;28(9):501-506.

ONO-2808-03 study design (NCT05923866)



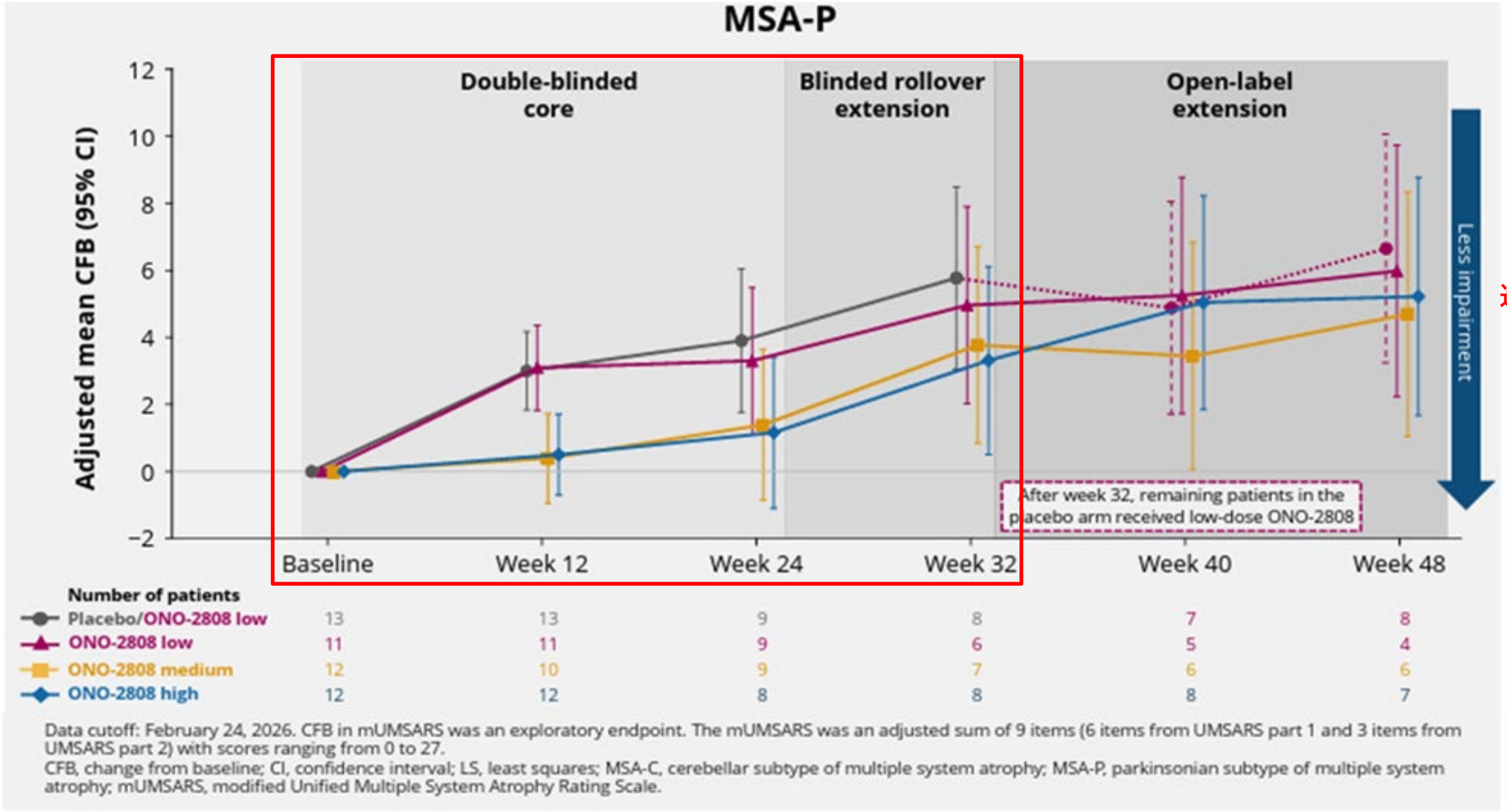
MSA, multiple system atrophy; MSA-C, cerebellar subtype of MSA; MSA-P, parkinsonian subtype of MSA.

Baseline demographics and clinical characteristics

Characteristic	Placebo n = 23	ONO-2808 low n = 23	ONO-2808 medium n = 23	ONO-2808 high n = 23	ONO-2808 total n = 69
Age, years					
Mean (SD)	59.6 (6.7)	63.2 (7.8)	63.6 (4.5)	60.4 (7.7)	62.4 (6.9)
Median (range)	58 (51–76)	63 (46–80)	65 (54–74)	60 (49–73)	62 (46–80)
Sex, n (%)					
Male	15 (65)	9 (39)	14 (61)	13 (57)	36 (52)
Female	8 (35)	14 (61)	9 (39)	10 (43)	33 (48)
Race, n (%)					
White	15 (65)	18 (78)	13 (57)	15 (65)	46 (67)
Asian	7 (30)	3 (13)	9 (39)	6 (26)	18 (26)
Other[a]	1 (4)	2 (9)	1 (4)	2 (9)	5 (7)
Years since diagnosis, n (%)					
0 to <1	8 (35)	6 (26)	10 (43)	8 (35)	24 (35)
1 to <2	10 (43)	10 (43)	6 (26)	9 (39)	25 (36)
2 to <3	3 (13)	4 (17)	3 (13)	2 (9)	9 (13)
3 to <4	2 (9)	2 (9)	3 (13)	3 (13)	8 (12)
4 to <5	0	1 (4)	1 (4)	1 (4)	3 (4)
MSA subtype, n (%)					
MSA-P	13 (57)	11 (48)	12 (52)	12 (52)	35 (51)
MSA-C	10 (43)	12 (52)	11 (48)	11 (48)	34 (49)

[a] Includes Black/African American and American Indian/Alaska Native.
MSA, multiple system atrophy; MSA-C, cerebellar subtype of MSA; MSA-P, parkinsonian subtype of MSA; SD, standard deviation.

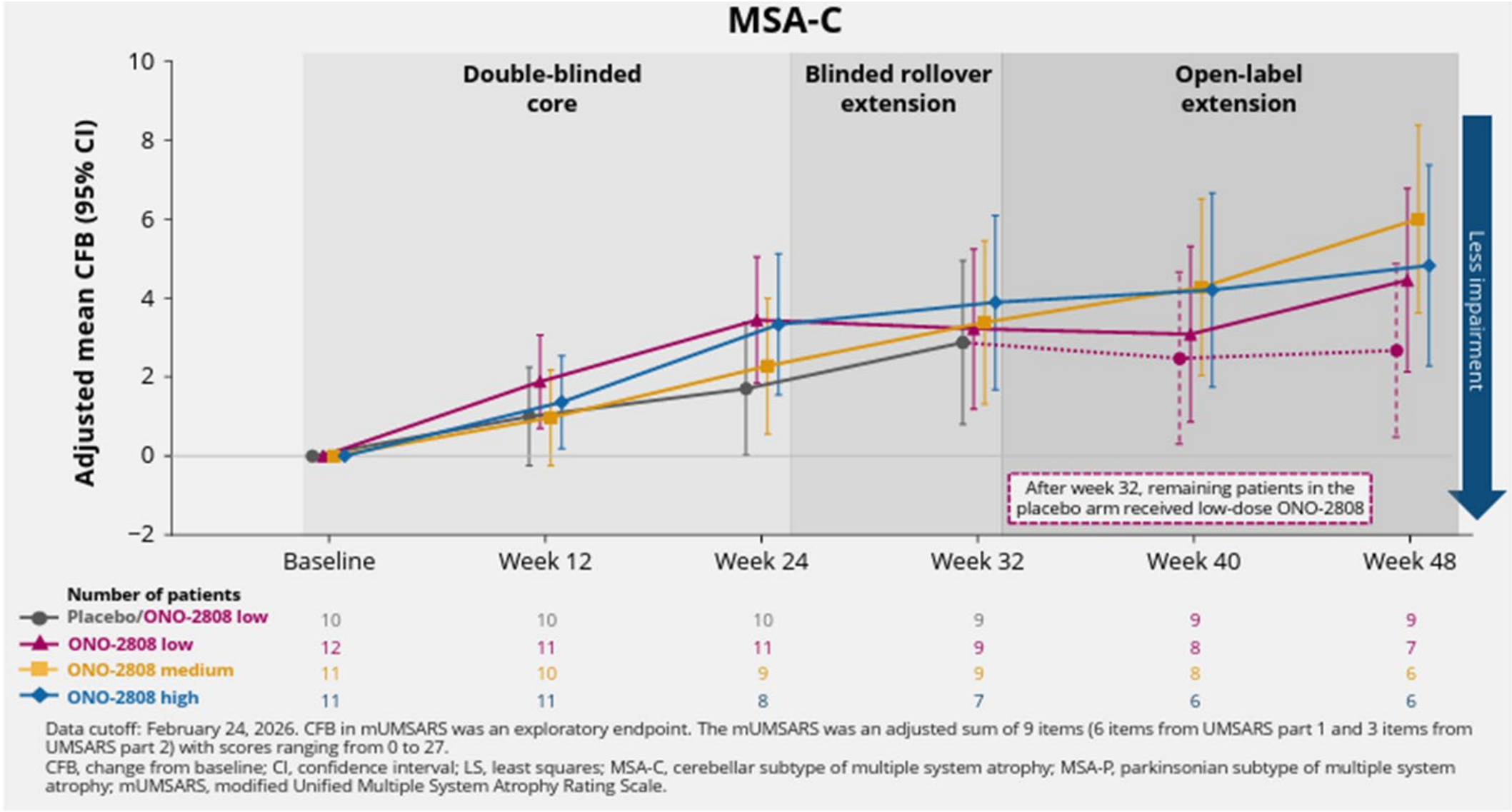
Efficacy-mUMSARS / MSA-P group



Data cutoff: February 24, 2026. CFB in mUMSARS was an exploratory endpoint. The mUMSARS was an adjusted sum of 9 items (6 items from UMSARS part 1 and 3 items from UMSARS part 2) with scores ranging from 0 to 27.
CFB, change from baseline; CI, confidence interval; LS, least squares; MSA-C, cerebellar subtype of multiple system atrophy; MSA-P, parkinsonian subtype of multiple system atrophy; mUMSARS, modified Unified Multiple System Atrophy Rating Scale.

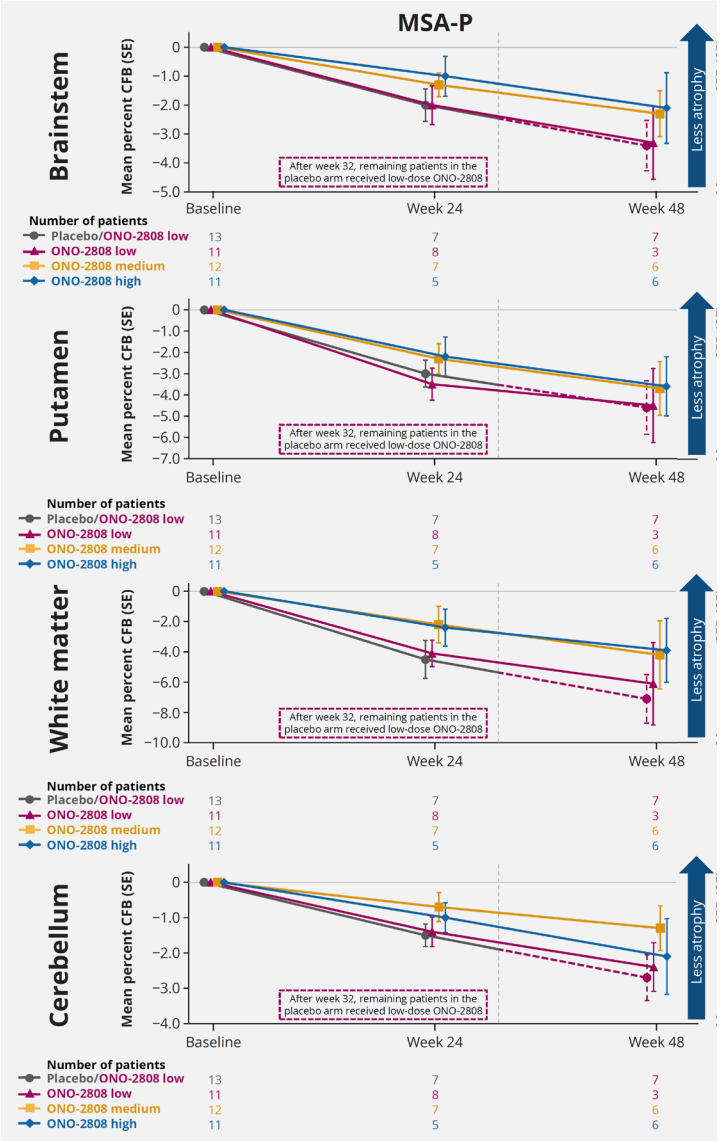
進行抑制

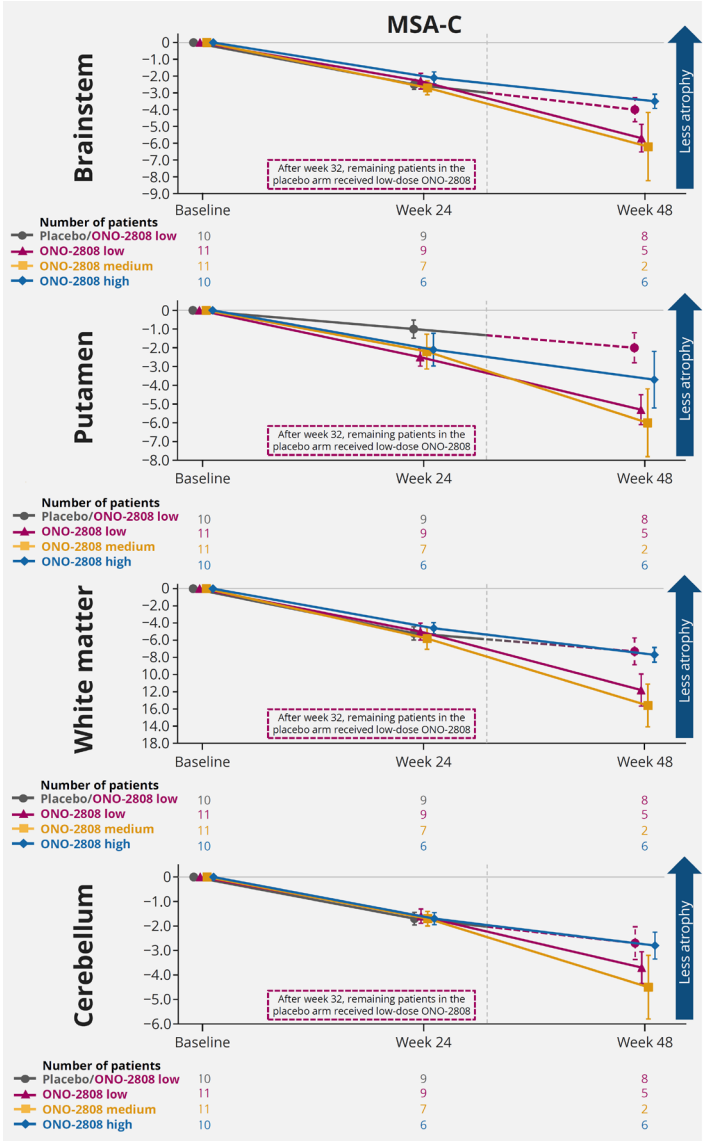
Efficacy-mUMSARS / MSA-C group



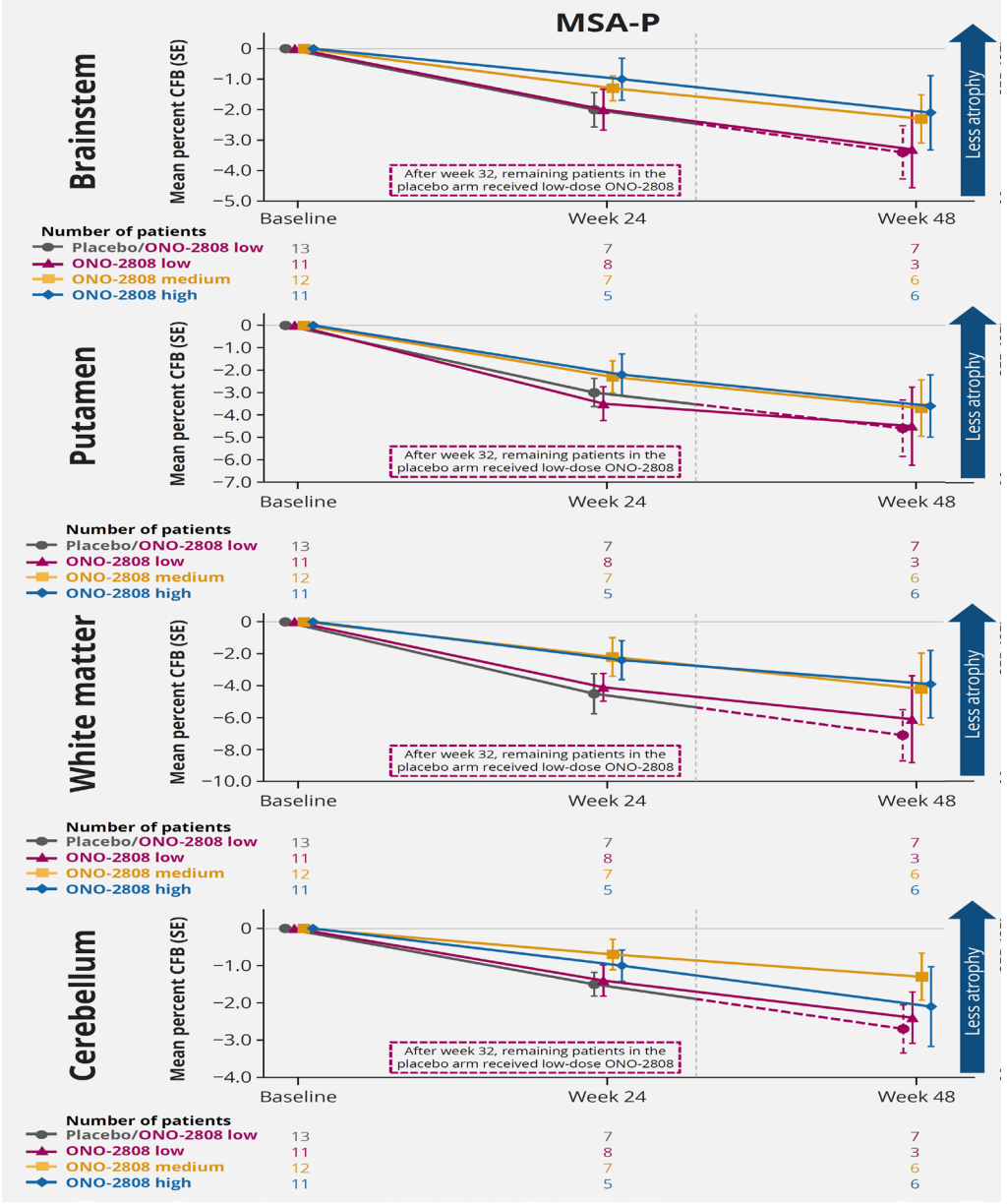
進行抑制

Efficacy-vMRI measurements

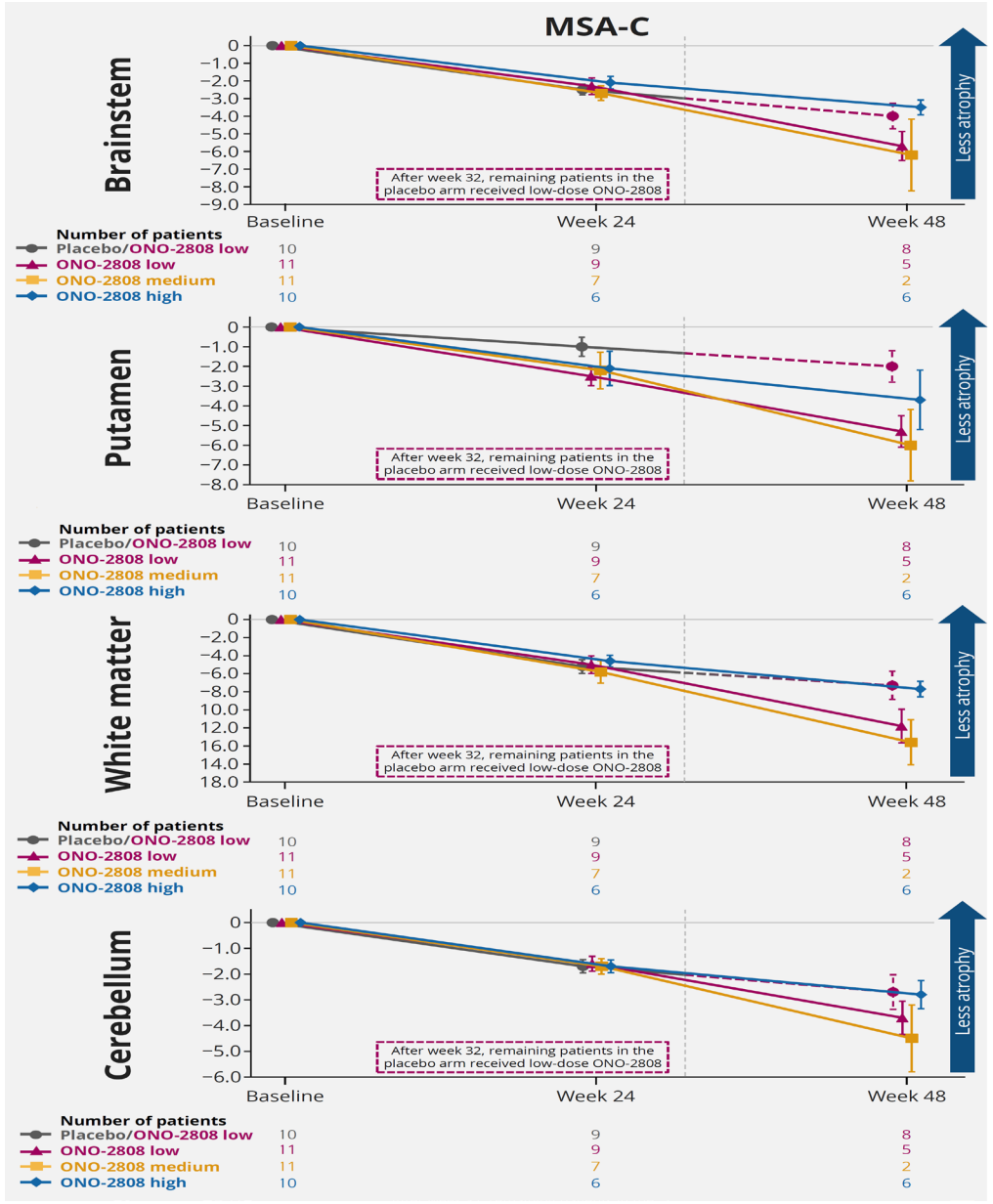




Efficacy-vMRI measurements / MSA-P group



Efficacy-vMRI measurements / MSA-C group



TEAEs in ≥10% of patients in any treatment group during the double-blinded core part

Preferred term, n (%)	Placebo n = 23	ONO-2808 low n = 23	ONO-2808 medium n = 23	ONO-2808 high n = 23	ONO-2808 total n = 69
Urinary tract infection	8 (35)	9 (39)	2 (9)	4 (17)	15 (22)
Headache	0	3 (13)	2 (9)	6 (26)	11 (16)
Constipation	0	1 (4)	0	4 (17)	5 (7)
Fall	3 (13)	3 (13)	2 (9)	0	5 (7)
Nasopharyngitis	1 (4)	0	2 (9)	3 (13)	5 (7)
Contusion	4 (17)	2 (9)	1 (4)	1 (4)	4 (6)
Fatigue	3 (13)	1 (4)	1 (4)	2 (9)	4 (6)
Arthralgia	4 (17)	1 (4)	1 (4)	1 (4)	3 (4)
Dizziness	1 (4)	0	0	3 (13)	3 (4)
Diarrhea	3 (13)	1 (4)	1 (4)	0	2 (3)
Skin abrasion	3 (13)	0	0	0	0

Data from the double-blinded core part , which included results with 24 weeks of follow-up. Adjudicated transaminase elevation events are described in Table 3.
TEAE, treatment-emergent adverse event.

Transaminase elevations adjudicated by the IDMC at week 24

Category, n (%)	Placebo n = 23	ONO-2808 low n = 23	ONO-2808 medium n = 23	ONO-2808 high n = 23	ONO-2808 total n = 69
Any transaminase elevation confirmed by IDMC	0	1 (4)	3 (13)	4 (17)	8 (12)
Any treatment-related transaminase elevation by highest severity					
Mild	0	0	1 (4)	2 (9)	3 (4)
Moderate	0	0	1 (4)	2 (9)	3 (4)
Severe	0	1 (4)	1 (4)	0	2 (3)
Any treatment-related transaminase elevation leading to withdrawal of treatment	0	1 (4)	3 (13)	3 (13)	7 (10)
Any serious transaminase elevations	0	1 (4)	0	0	1 (1)
Treatment-related	0	1 (4)	0	0	1 (1)

Data from the double-blinded core part, which included results with 24 weeks of follow-up.
IDMC, independent data monitoring committee.

ONO-2808-03試験の結果をふまえて

Modified UMSARS for ONO-2808

ONO-2808-03試験で使用（最大値：27点）

-	パートI：既往歴評価（日常生活動作） 患者・介護者への聞き取りにより、過去2週間の平均的な機能を評価	パートII：運動検査尺度（運動機能評価） 最も症状の重い肢で評価
1	発話	表情
2	嚥下	発話
3	書字	眼球運動障害
4	食物を切ることと食事用具の取扱い	安静時振戦
5	更衣	動作時振戦
6	衛生	筋緊張亢進
7	歩行	手のすばやい交互運動
8	転倒	指のタッピング
9	起立に伴う症状	下肢の敏捷性
10	排尿機能	踵膝脛試験
11	性機能	椅子からの立ち上がり
12	排便機能	姿勢
13	---	身体の動揺
14	---	歩行
-	最小0点、最大18点	最小0点、最大9点

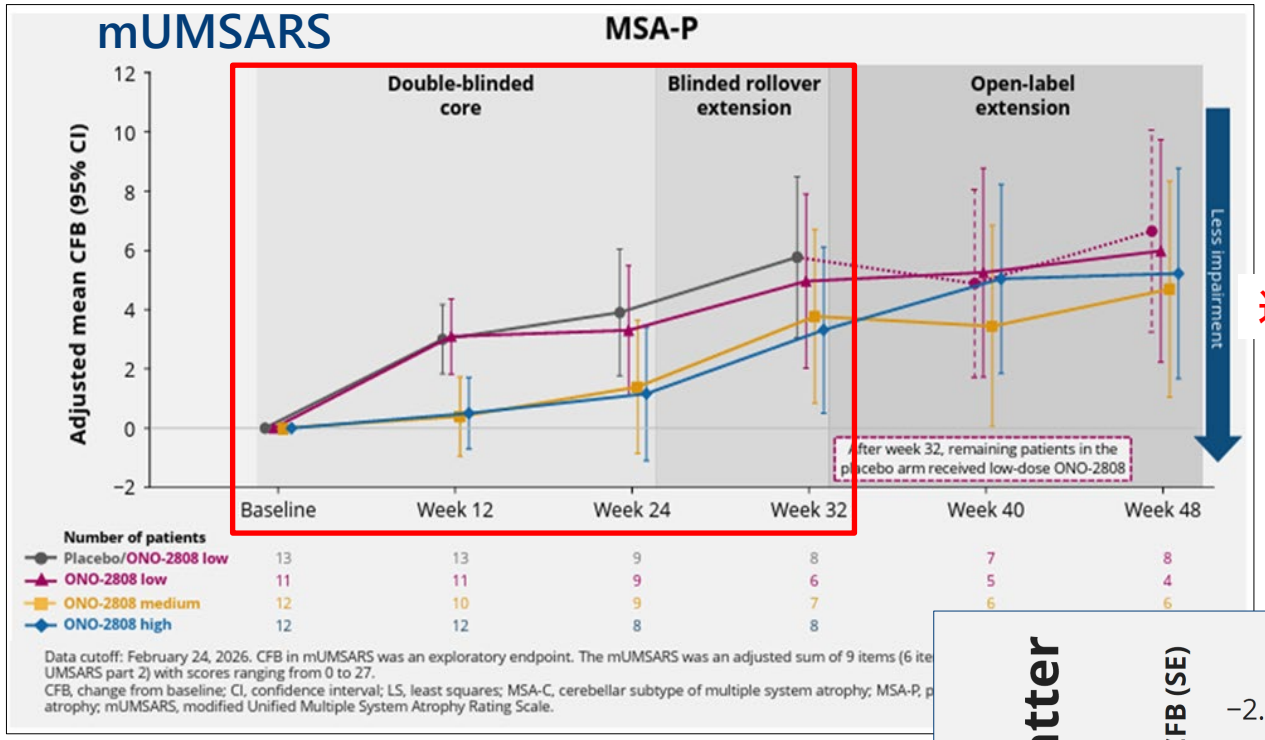
Modified UMSARS for Amlenetug (Lu AF82422)

Lundbeck社先行品（Amlenetug, Lu AF82422）がP2で使用（最大48点）

-	パートI：既往歴評価（日常生活動作） 患者・介護者への聞き取りにより、過去2週間の平均的な機能を評価	パートII：運動検査尺度（運動機能評価） 最も症状の重い肢で評価
1	発話	表情
2	嚥下	発話
3	書字	眼球運動障害
4	食物を切ることと食事用具の取扱い	安静時振戦
5	更衣	動作時振戦
6	衛生	筋緊張亢進
7	歩行	手のすばやい交互運動
8	転倒	指のタッピング
9	起立に伴う症状	下肢の敏捷性
10	排尿機能	踵膝脛試験
11	性機能	椅子からの立ち上がり
12	排便機能	姿勢
13	---	身体の動揺
14	---	歩行
-	最小12点、最大48点	0点

Kjærsgaard L et al., Lancet Neurol. 2026 Jun;25(6):560-570.より解釈

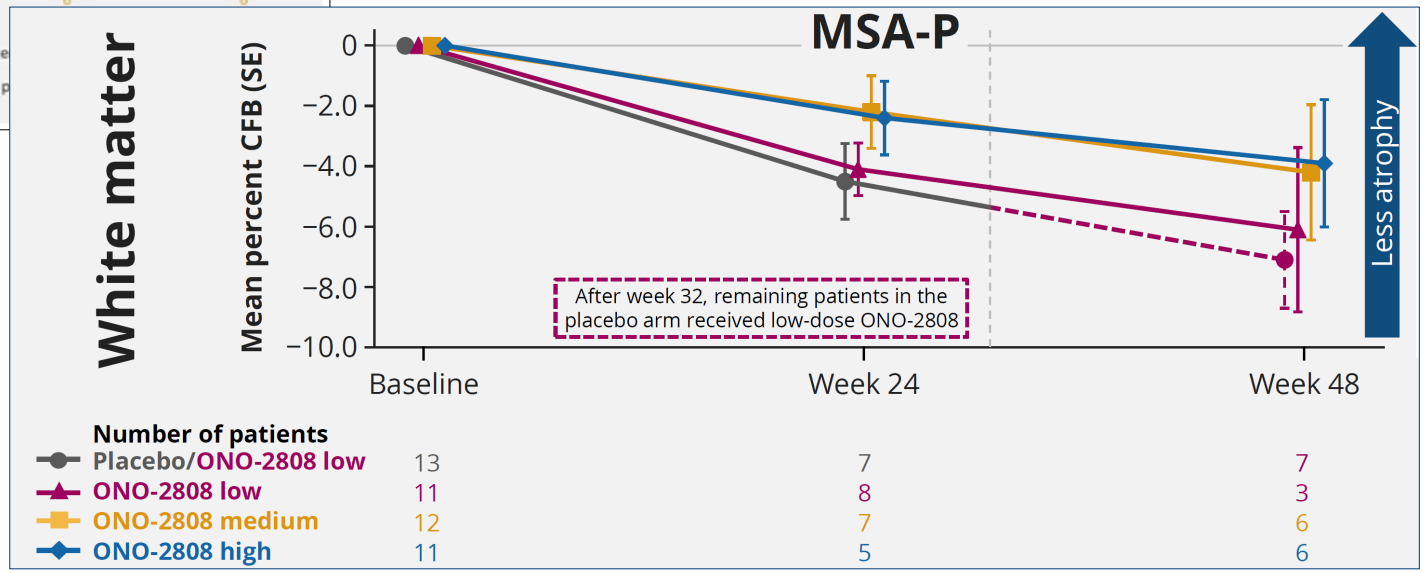
ONO-2808 Summary



進行抑制

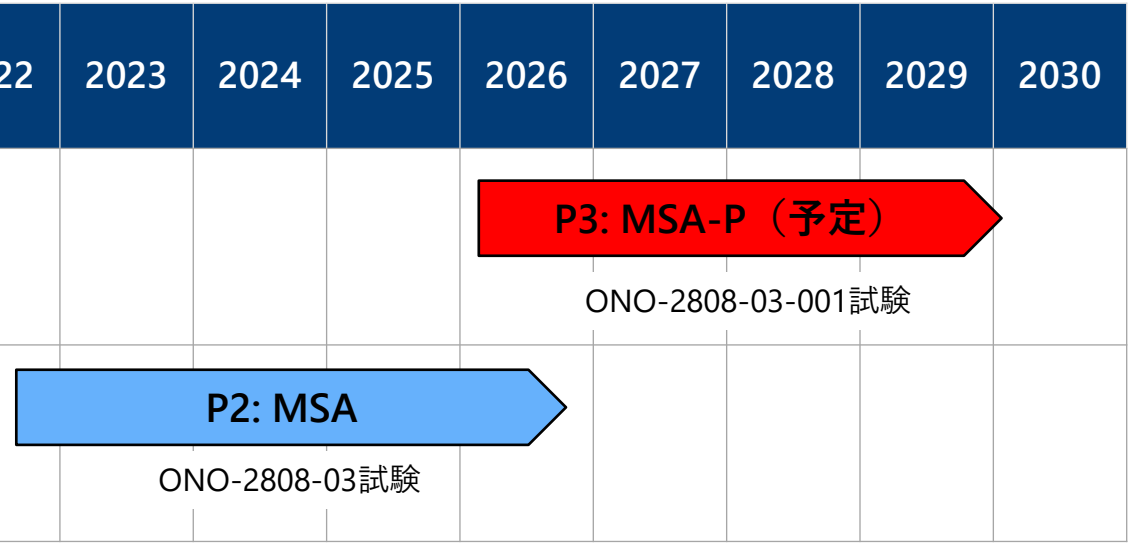
vMRI measurements

脳萎縮抑制



ONO-2808 開発状況

適応症等	開発相	進捗	実施国	試験番号
MSA（MSA-P）	PIII	開始前	日米欧など予定（未発表）	未定
MSA	P II	2025年度主要データ取得済み	日米	NCT05923866



本日のアジェンダ

オープニング

POC試験のデータ詳細

- ONO-4578（ASCO2026）
- ONO-2808（7th 世界パーキンソン病学会）

中枢神経領域における創薬活動

滝野　十一

代表取締役社長　COO

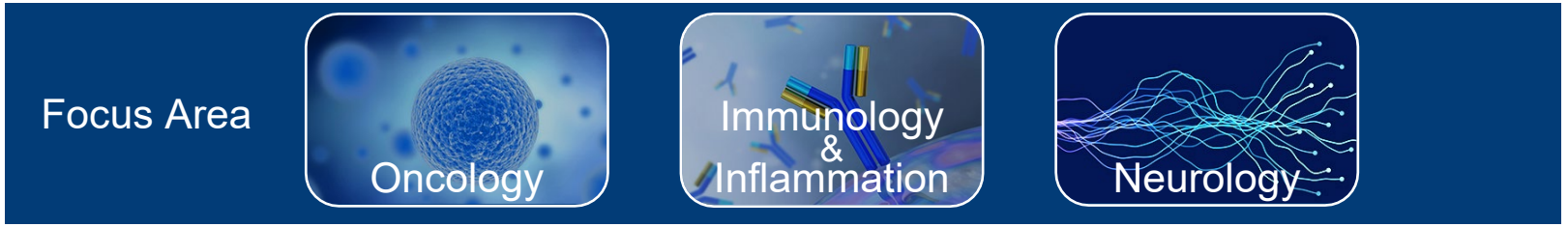
岡本　達也

執行役員　開発本部長

勝又　清至

執行役員　研究本部長

小野薬品の創薬



ONO PHARMA UK LTD.
London since 1998
◆ Discovery Research Alliance Activities

ONO PHARMA USA, INC.
Cambridge, MA since 1998
◆ Discovery Research Alliance Activities

ONO PHARMACEUTICAL CO.,LTD.
Japan

deciphera Deciphera Pharmaceuticals, founded in 2003
Lawrence, KA since 2024
◆ Research Institute

ONO VENTURE INVESTMENT, INC.
South San Francisco, CA since 2020
◆ Investment in startup companies

- 研究・創薬提携実施数：国内外で
  約120件（2026年3月末時点の稼働数）

➢ 世界中のアカデミア等との協業による、新たな創薬シーズの探索
➢ 最新の技術を持つバイオテック等との協業による、最適モダリティでの創薬

神経領域における創薬戦略

- ✓ 未充足の医療ニーズに応えるために、疾患バイオロジーを深掘りする
- ✓ 症状改善薬だけでなく、疾患修飾薬も目指す
- ✓ 神経細胞だけでなく、グリア細胞も制御する
- ✓ 臨床の知見を活かして、ヒト外挿性を高める

最新技術とオープンイノベーションで、新薬を届ける

神経領域におけるパイプラインの変遷

AD　：アルツハイマー型認知症
ALS　：筋萎縮性側索硬化症
CIPN　：化学療法誘発末梢神経障害
CINV　：抗がん剤投与に伴う悪心・嘔吐
DPN　：糖尿病性末梢神経障害
MSA　：多系統萎縮症
PD　：パーキンソン病

神経変性

CATACLOT®
くも膜下出血、脳血栓症

ONO-1603
AD

ONO-2506
AD、PD、ALS、脳梗塞急性期
（Merckへ導出）

RIVASTACH®
AD
(Novartisから導入)

ONO-4641
多発性硬化症
（Merck Seronoへ導出）

ONO-2160
PD

ONGENTYS®
PD
（Bialから導入）

精神・神経など

ONO-2333MS
うつ病

ONO-2745
短時間作用型全身麻酔
（Paionから導入）

ONO-2909
ナルコレプシー

疼痛・神経障害

KINEDAK®
DPN

ONO-9902
疼痛

OPALMON®
脊柱管狭窄症

EMEND®
CINV
（Merckから導入）

ONO-2921
神経障害性疼痛

ONO-2952
過敏性腸症候群

ONO-2910
CIPN、DPN

開発中

ONO-2020
AD、ADに伴うアジテーション
P2実施中

ONO-2808
MSA
POC確立

ONO-2017
てんかん**申請中**
（SK bioから導入）

ONO-1110
うつ病、社交不安症
P2実施中

ONO-2416
精神疾患
P1実施中

ONO-1110
帯状疱疹後神経痛、線維筋痛症、
ハンナ型間質性膀胱炎
P2実施中

小野薬品のグリア創薬

AD　：アルツハイマー型認知症
CIPN：化学療法誘発末梢神経障害
DPN　：糖尿病性末梢神経障害
MSA　：多系統萎縮症

他社に先んじてグリア創薬に着手

ONO-2506（アロサイト注、セレアクト）
の研究開発

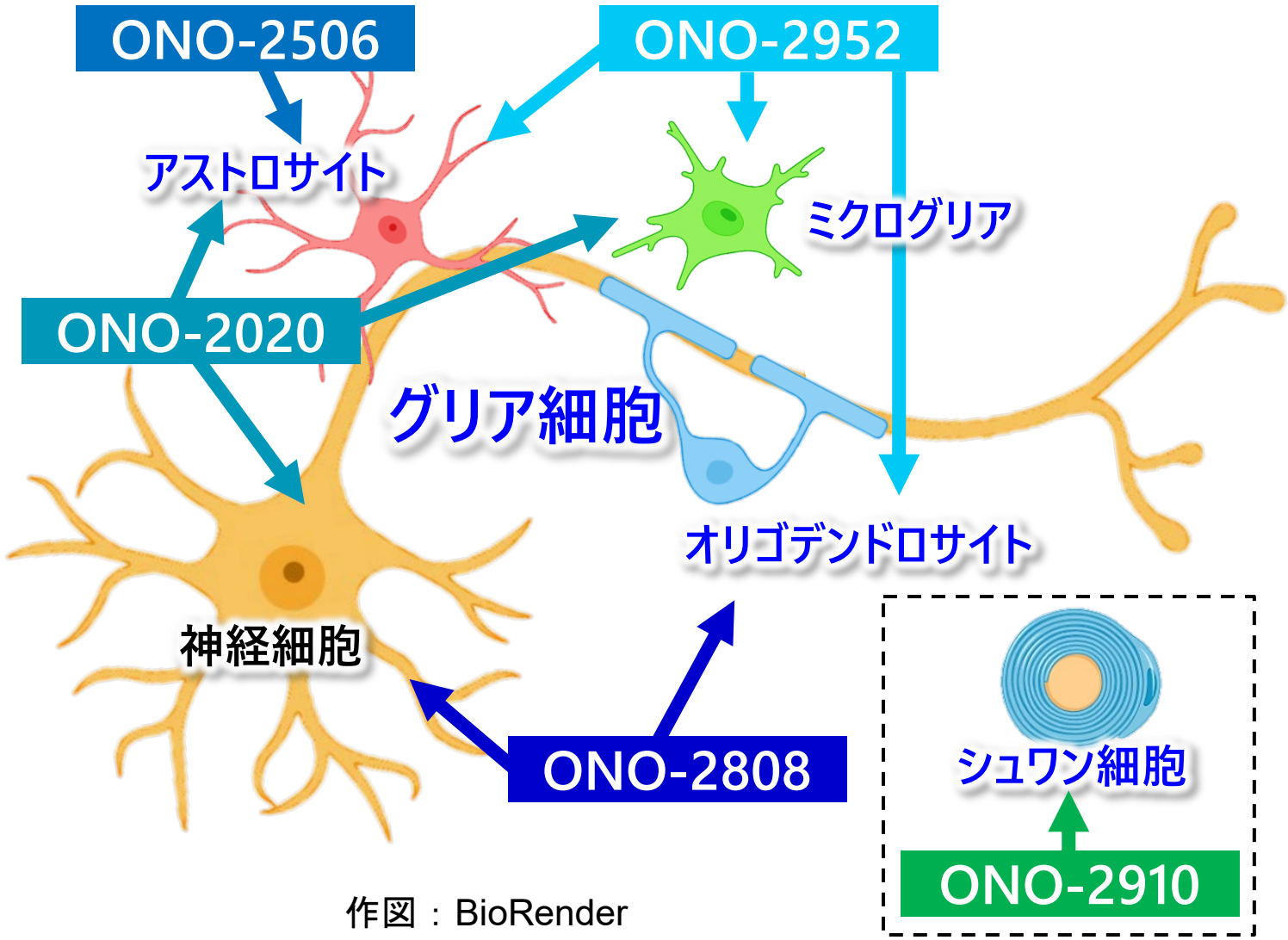
専門家ネットワークを活かして創薬推進

ONO-2952：TSPO拮抗薬
（ストレス疾患、過敏性腸症候群）

ONO-2910：シュワン細胞分化促進薬
（CIPN、DPN）

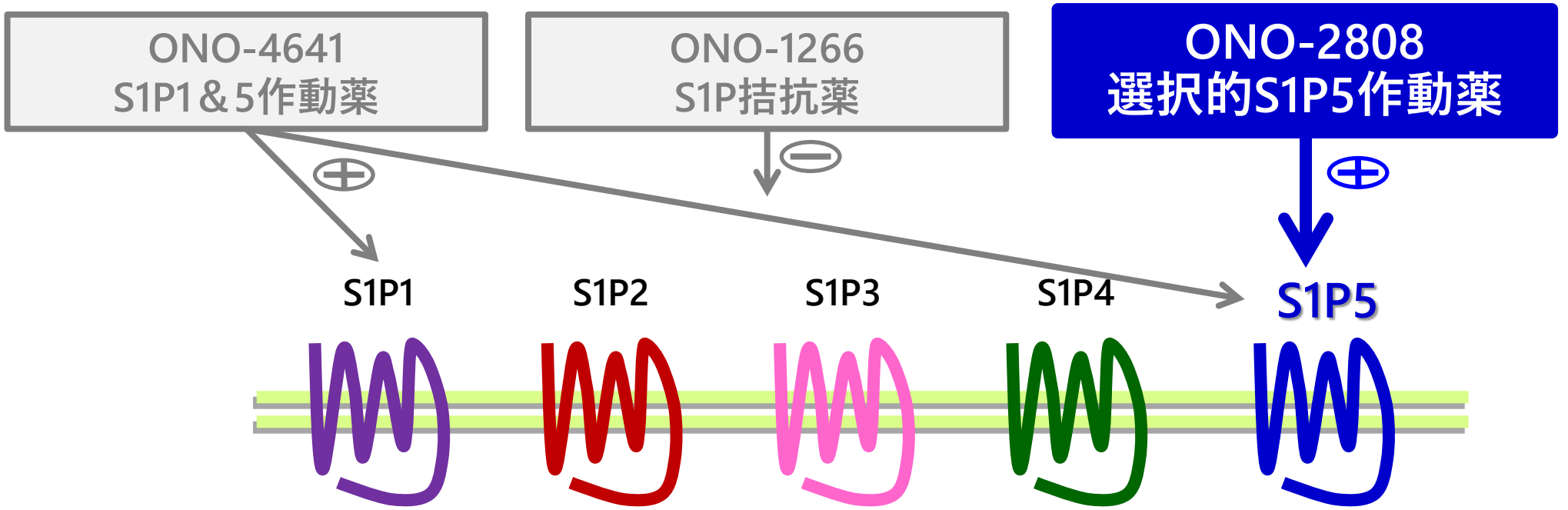
◆ ONO-2808：S1P5作動薬
（MSA）

◆ ONO-2020：エピジェネティクス制御薬
（AD、ADに伴うアジテーション）



作図：BioRender

小野薬品の脂質創薬（スフィンゴシン-1-リン酸受容体制御）



- スフィンゴシン-1-リン酸（S1P）の機能に着目した創薬
- ONO-2808は、オリゴデンドロサイトや神経細胞への作用に着目

小野薬品のイオンチャネル創薬

オープンイノベーションによる技術の活用

- BioFocus (現Charles River)
- Evotec
- Vanderbilt University
- Xention (現Metrion Biosciences)

様々なイオンチャネルに対する創薬に挑戦

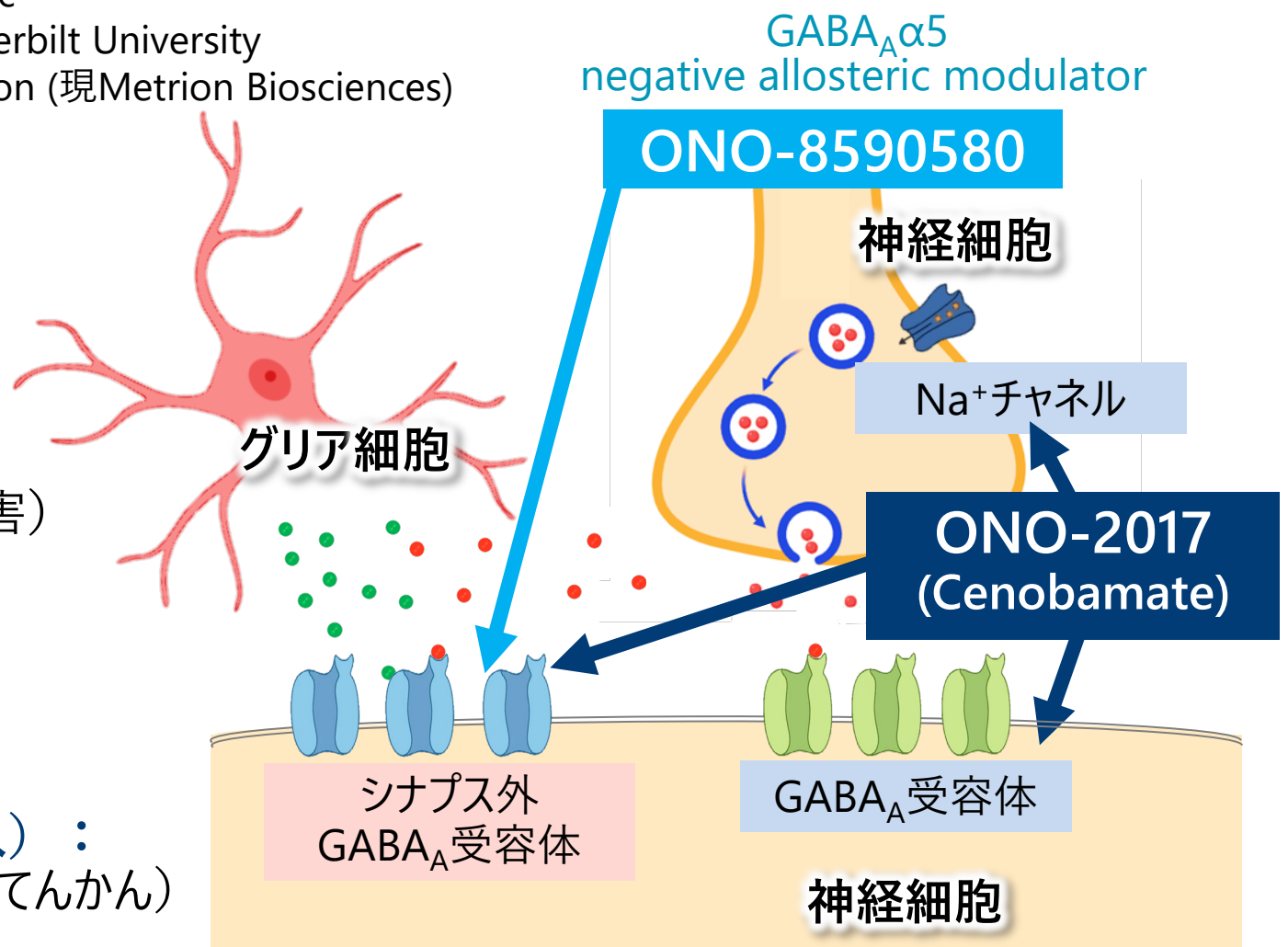
◆ **$GABA_A$α5 NAM**（認知機能障害）

◆ **イオンチャネルA 阻害薬**（疼痛）

◆ **イオンチャネルB 制御薬**（疼痛、認知機能障害）

シナプス外$GABA_A$受容体への作用に着目

◆ **ONO-2017（Cenobamate、SK bioから導入）：電位依存性Na^+チャネル阻害+$GABA_A$活性化**（てんかん）

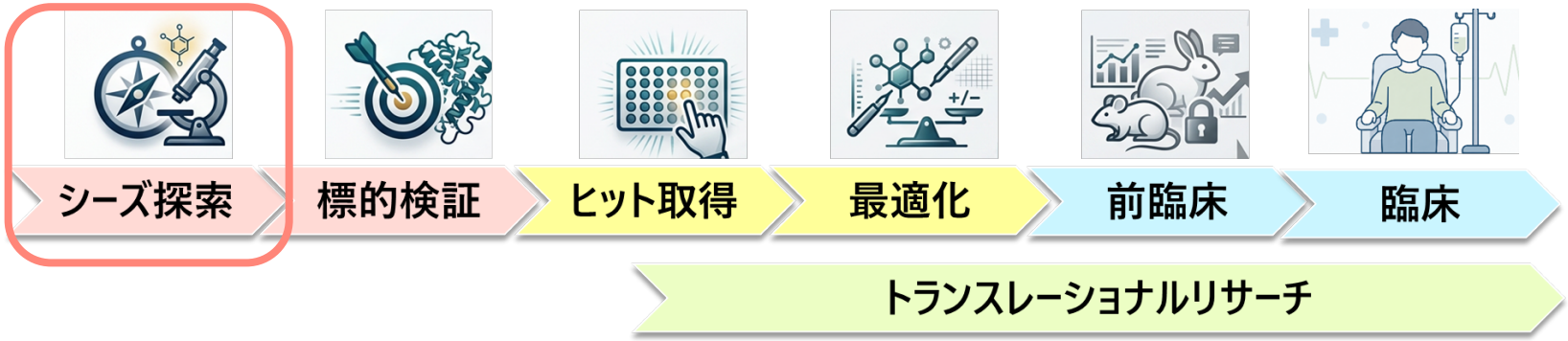


作図：BioRender

神経領域の創薬における課題と対策

課題	対策
標的妥当性 ● 病態の理解 ● 脳や脳脊髄液の入手 ● 適切な疾患モデル	✓ 共同研究による創薬標的探索 ✓ アカデミアの有する病態を再現した評価系の活用
モノづくり ● 良質なヒット取得 ● 中枢移行性	✓ バイオテックとの協業による創薬プラットフォームの活用 ✓ アカデミア／バイオテックの計算科学能力の活用
ヒトへの外挿 ● 臨床有効性予測 ● 臨床試験での適切な評価	✓ 創薬初期からバイオマーカー探索、利用 ✓ 臨床研究によるバイオマーカー探索

神経領域の創薬における課題と対策：シーズ探索



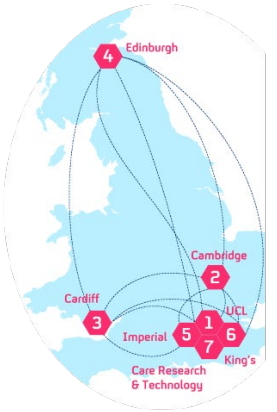
東北大学東北メディカル・メガバンク機構（ToMMo）

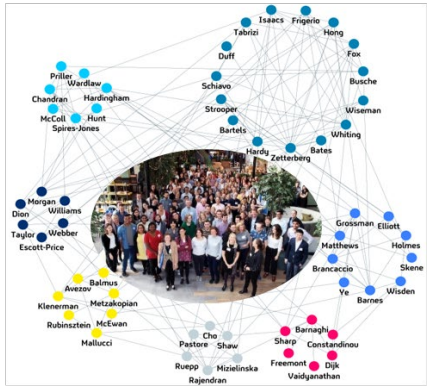
- 2013年から開始された地域住民コホート調査および三世代コホート調査
- 追跡も可能とした一般住民の大規模全ゲノム解析
- 2024年6月、日本人10万人の全ゲノム解析が完了（世界有数の規模）
- 小野は、2021年3月より統合解析コンソーシアムに参画

TOHOKU MEDICAL MEGABANK ORGANIZATION

世界規模のゲノム解析結果から
標的妥当性を検証

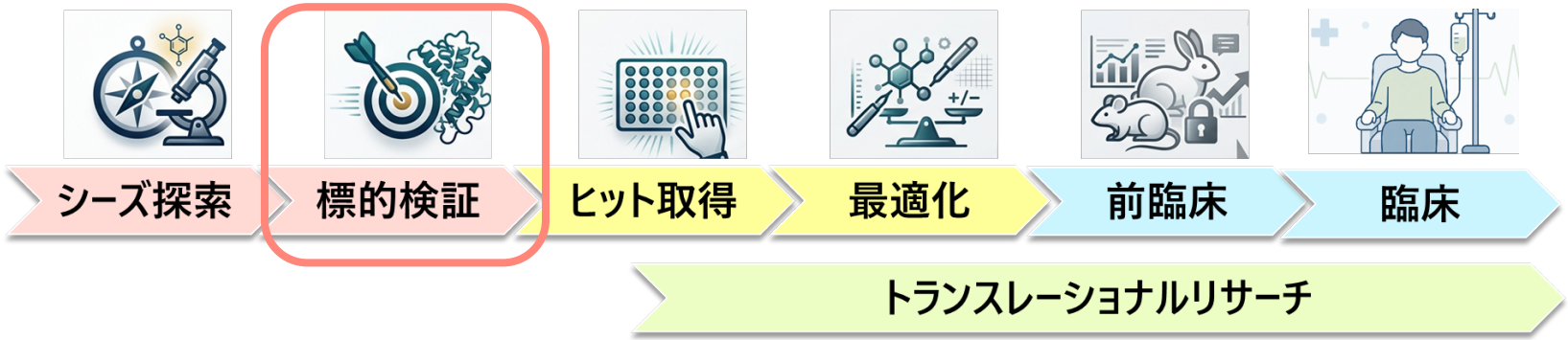
UK Dementia Research Instituteとの共同研究





英国での研究ネットワークと高い研究力から
新しい創薬の標的を同定

神経領域の創薬における課題と対策：標的検証

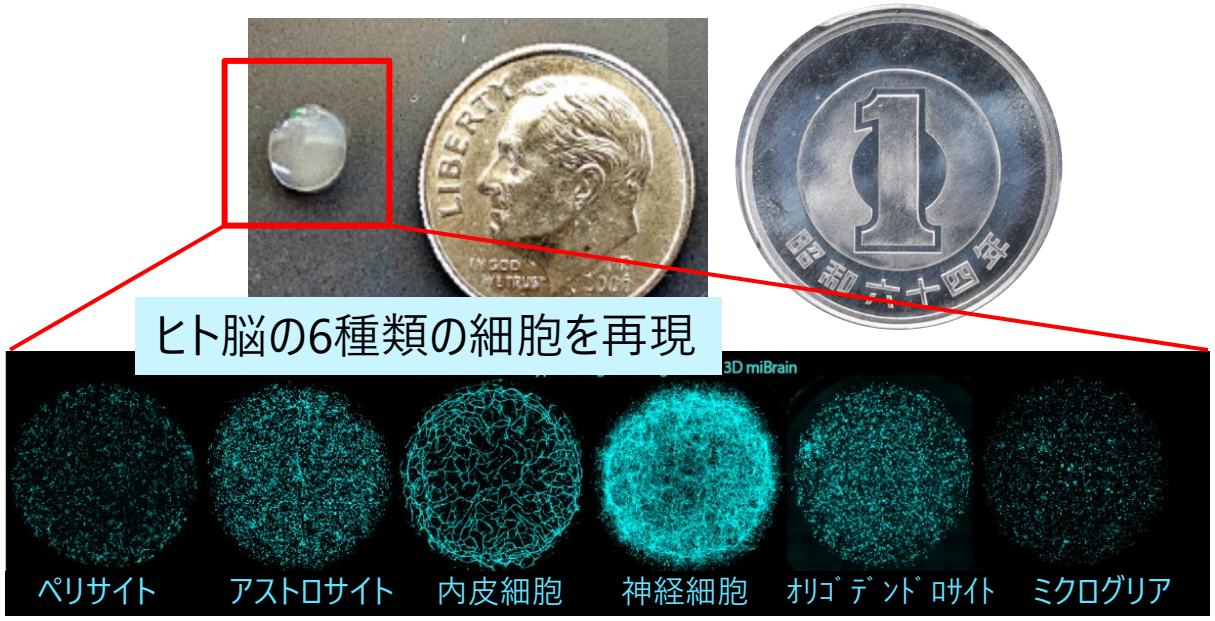


まほろロボットの導入

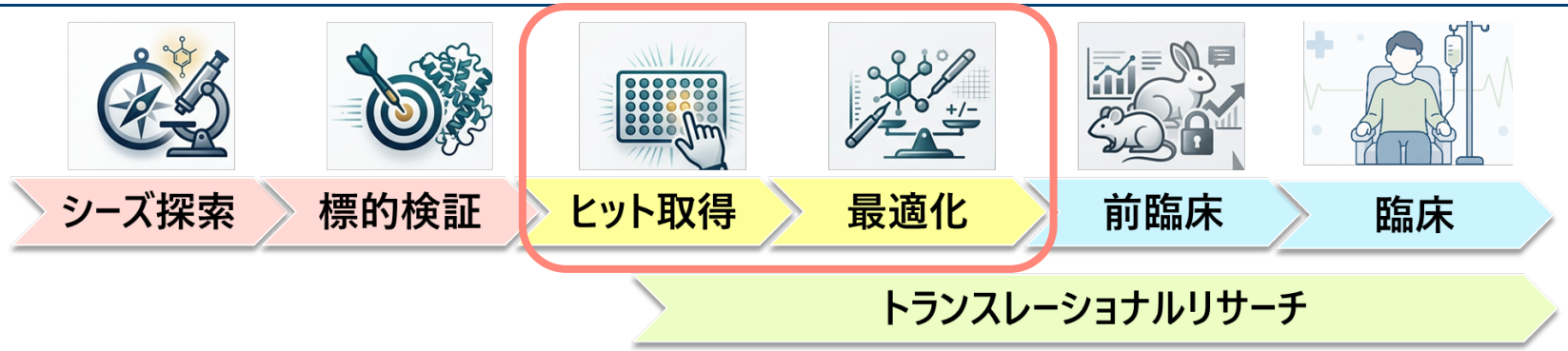
iPS細胞を安定培養し、創薬プロジェクトに活用

iPS細胞を用いた脳オルガノイドモデル

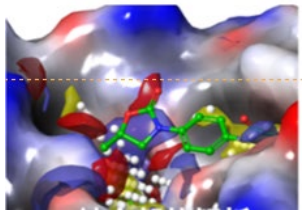
脳ｵﾙｶﾞ ﾉｲﾄﾞ　10セント　1円玉



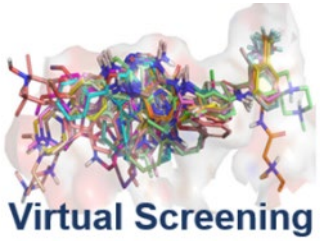
神経領域の創薬における課題と対策：ヒット取得・最適化



創薬スパコン「Tokyo-1」によりAI創薬を推進



結合部位解析



大量の化学データや生物化学的情報を
高速で処理・分析し、良質なヒット化合物を取得

Vanderbilt Universityとの創薬提携

December 10, 2015

Vanderbilt, Ono Pharmaceutical sign drug discovery agreement

Vanderbilt University Medical Center and Ono Pharmaceutical Group, an international company based in Japan, have signed a drug discovery agreement.

https://news.vumc.org/2015/12/10/vanderbilt-ono-pharmaceutical-sign-drug-discovery-agreement/

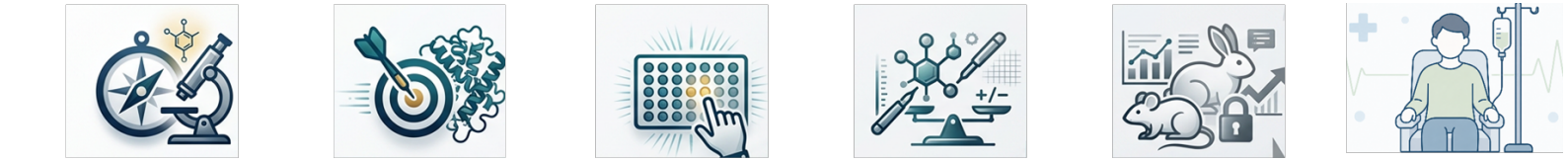
Lindsley Lab

WARREN CENTER FOR NEUROSCIENCE DRUG DISCOVERY

https://lab.vanderbilt.edu/lindsleylab/

大学のイオンチャネル創薬のノウハウを活かし、
迅速に化合物を最適化

神経領域の創薬における課題と対策：トランスレーショナルリサーチ



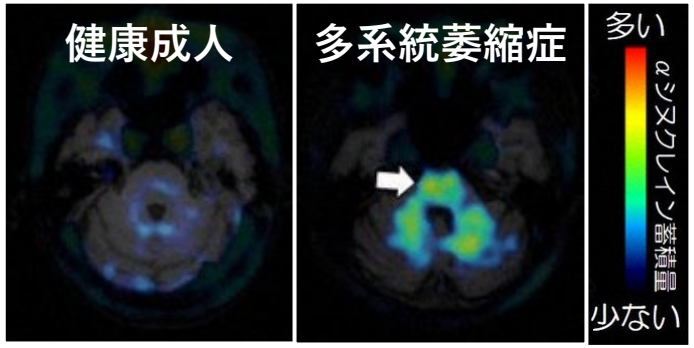
脳PETリガンド共同開発（脳疾患創薬アライアンス）

αシヌクレイン病変
PETの共同開発

調和ある多様性の創造 国立研究開発法人 QST 量子科学技術研究開発機構 National Institutes for Quantum Science and Technology
Eisai ono Takeda

▼

ヒトで脳内αシヌクレイン病変の画像化に成功（世界初）



'22年8月31日量子研PRより
（https://www.qst.go.jp/site/press/20220831.html）

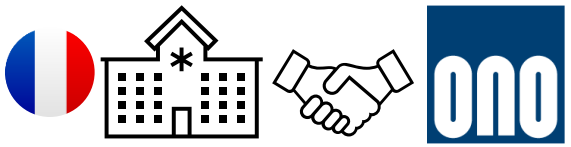
疾患の縦断的臨床研究（医師主導観察研究）

Disease Progression in Multiple System Atrophy: The ASPIRE Multi-Modal Biomarker Study

Margherita Fabbri, Natalia del Campo, Wassilios G. Meissner, Vanessa Rousseau, Agnès Sommet, Pierre Payoux, Pierre Gantet, Amel Drif, Hélène Catalá, Claire Thalamas, Christine Tranchant, Franck Durif, Ana Marques, Alexandre Eusebio, Luc Defebvre, Jean-Christophe Corvol, Stéphane Thobois, Anthime Flaus, Anne-Gaelle Corbille, Solène Frismand, Beverley Patterson, Alexandra Foubert-Samier, Anne Pavy-Le Traon, Germain Arribarat, Patrice Péran, PhD, and Olivier Rascol, MD, PhD, for the ASPIRE Study Group

Ann Neurol. 2026 Jan;99(1):96-113.

フランス・Toulouse大学と提携



多系統萎縮症に関する
臨床バイオマーカーの同定

▼

ONO-2808の
臨床試験に活用

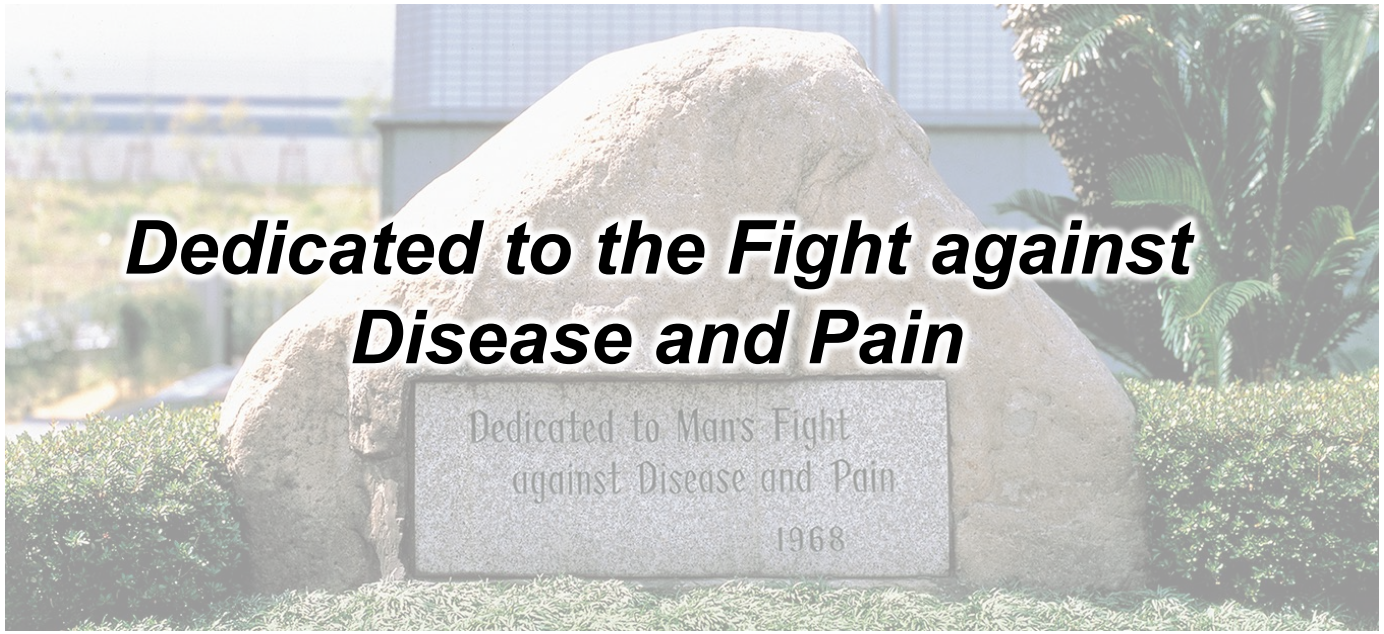
臨床パイプライン（神経領域）

開発コード	適応症	PⅠ	PⅡ	PⅢ	申請	承認
ONO-2017	てんかん部分発作	■	■	■	■	
	てんかん部分発作(小児)	■	■	■		
	てんかん強直間代発作	■	■	■		
ONO-2808	多系統萎縮症	■	■			
ONO-1110	帯状疱疹後神経痛	■	■			
	線維筋痛症	■	■			
	ハンナ型間質性膀胱炎	■	■			
	うつ病	■	■			
	社交不安症	■	■			
ONO-2020	アルツハイマー型認知症 (AD)	■	■			
	ADに伴うアジテーション	■	■			
ONO-2416	精神疾患	■				

⋮ Coming soon

まとめ

- 創薬の各プロセスにおいて社内外の知見を組み合わせ、パイプラインを強化している
- 神経疾患だけでなく、がん、免疫・炎症性疾患の未充足の医療ニーズに応えるべく、最新技術とオープンイノベーションをフル活用し、一日も早く世界の患者さんに革新的な新薬を届けるよう挑戦し続ける



小野薬品工業株式会社

Dedicated to the Fight against Disease and Pain