RTP Live from Chicago: Investigator Perspectives on Available Research Findings and Challenging Questions in the Management of Soft Tissue Sarcoma and Other Connective Tissue Neoplasms

Tuesday, June 3, 2025
7:00 AM – 8:00 AM CT (8:00 AM – 9:00 AM ET)

Faculty

Rashmi Chugh, MD
Mrinal Gounder, MD

Moderator

Neil Love, MD

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Faculty

Rashmi Chugh, MD
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Division of Hematology/Oncology
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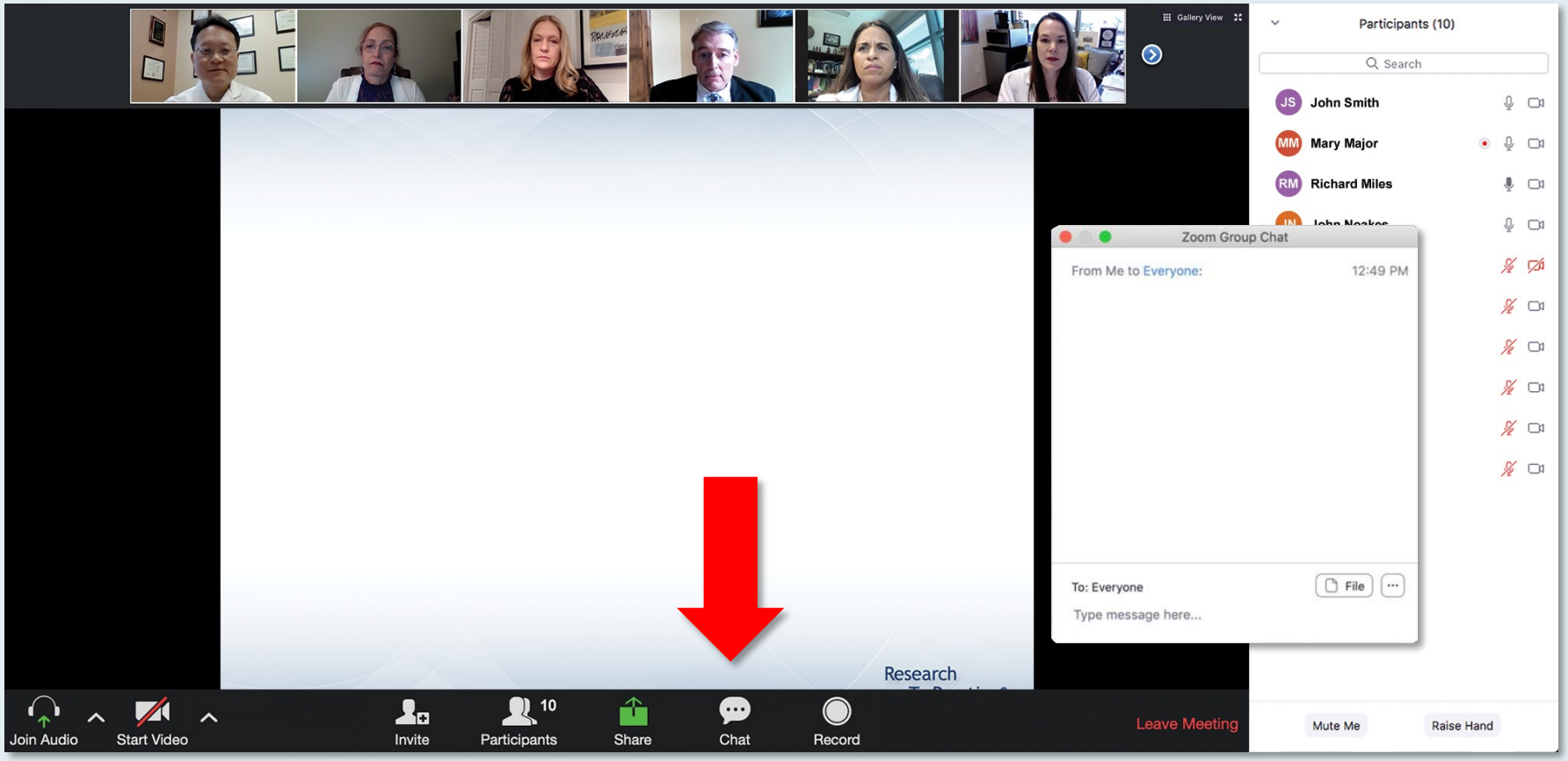
We Encourage Clinicians in Practice to Submit Questions



Feel free to submit questions now before the program begins and throughout the program.

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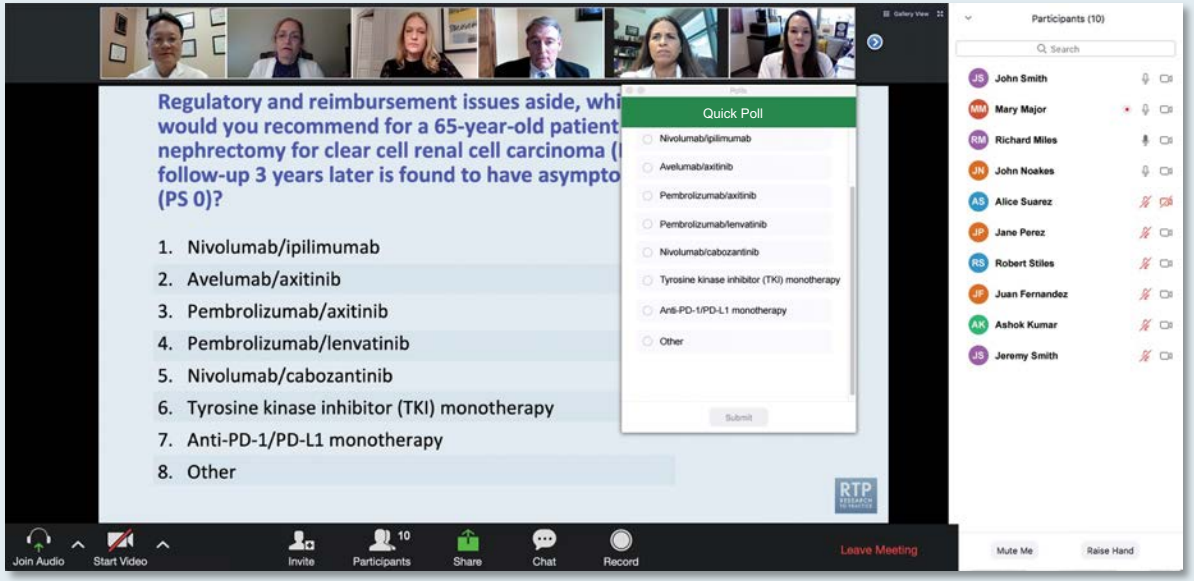
Clinicians in the Audience, Please Complete the Pre- and Postmeeting Surveys



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Friday May 30	**Immunotherapy and Antibody–Drug Conjugates in Lung Cancer** 11:15 AM – 12:45 PM CT (12:15 PM – 1:45 PM ET)
	Colorectal Cancer 6:30 PM – 8:30 PM CT (7:30 PM – 9:30 PM ET)
	EGFR Mutation–Positive Non–Small Cell Lung Cancer 6:30 PM – 8:30 PM CT (7:30 PM – 9:30 PM ET)
Saturday May 31	**Urothelial Bladder Cancer** 6:45 AM – 7:45 AM CT (7:45 AM – 8:45 AM ET)
	Non–Hodgkin Lymphoma 7:00 PM – 9:00 PM CT (8:00 PM – 10:00 PM ET)
	Prostate Cancer 7:00 PM – 9:00 PM CT (8:00 PM – 10:00 PM ET)
Sunday June 1	**Chronic Lymphocytic Leukemia (Webinar)** 7:00 AM – 8:00 AM CT (8:00 AM – 9:00 AM ET)
	HER2–Positive Gastrointestinal Cancers 7:00 PM – 8:30 PM CT (8:00 PM – 9:30 PM ET)
	Ovarian and Endometrial Cancer 7:00 PM – 9:00 PM CT (8:00 PM – 10:00 PM ET)
Monday June 2	**Renal Cell Carcinoma (Webinar)** 7:00 AM – 8:00 AM CT (8:00 AM – 9:00 AM ET)
	Multiple Myeloma (Webinar) 6:00 PM – 7:00 PM CT (7:00 PM – 8:00 PM ET)
	Metastatic Breast Cancer 7:00 PM – 9:00 PM CT (8:00 PM – 10:00 PM ET)
Tuesday June 3	**Soft Tissue Sarcoma and Other Connective Tissue Neoplasms (Webinar)** 7:00 AM – 8:00 AM CT (8:00 AM – 9:00 AM ET)

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Dr Chugh — Disclosures
Faculty

Advisory Committees	Deciphera Pharmaceuticals Inc, Inhibrx, Recordati Rare Diseases
Contracted Research	Adaptimmune, Astex Pharmaceuticals, Ayala Pharmaceuticals, Cogent Biosciences, Inhibrx, Kronos Bio, Pfizer Inc, PharmaMar, Polaris Pharmaceuticals

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Dr Gounder — Disclosures
Faculty

Consulting Agreements	Aadi Bioscience, Avacta Therapeutics, Ayala Pharmaceuticals, Epizyme Inc, Ikena Oncology, Kura Oncology, Orion Corporation, Parabilis Medicines, Rain Oncology, Regeneron Pharmaceuticals Inc, Syros Pharmaceuticals Inc
Contracted Research	Avacta Therapeutics, Ayala Pharmaceuticals, Ikena Oncology, Immunome, Ipsen Biopharmaceuticals Inc, Kura Oncology, Orion Corporation, Parabilis Medicines, Pyxis Oncology, Tango Therapeutics, Vivace Therapeutics
Data and Safety Monitoring Boards/Committees	Kura Oncology

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Dr Love — Disclosures

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Agenda

Introduction: Current Role of General Medical Oncologists in the Treatment of Soft Tissue Sarcomas (STS)

Module 1: Incorporation of Novel Agents and Strategies into the Management of STS — Faculty Presentation

Module 2: Incorporation of Novel Agents and Strategies into the Management of STS — Survey Questions

Module 3: Evolving Treatment Paradigm for Locally Aggressive STS — Faculty Presentation

Module 4: Evolving Treatment Paradigm for Locally Aggressive STS — Survey Questions

Module 5: ASCO 2025

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Module 5: ASCO 2025

Survey of 50 Community-Based General Medical Oncologists
May 14-24, 2025

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Number of Oncologists Treating Patients with…

		Median (Range)
Soft tissue sarcomas	46 (94%)	3 (0-60)
Desmoid tumors	22 (44%)	0 (0-10)
Tenosynovial giant cell tumor (TGCT)	8 (16%)	0 (0-2)

Questions from General Medical Oncologists — Role of GMOs in STS Treatment

- Our large tertiary institution that sees sarcoma does not take many insurances and can be challenging to get them into multidisciplinary providers. In rural community virtual evals may be of benefit particularly if surgical therapy is not front-line therapy.
- I think the biggest impediment is lack of large numbers of patients with these diseases, need for collaboration with experts but they can be difficult to access.

Agenda

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Research to Practice

ASCO Soft Tissue Sarcoma Live Webinar

Incorporation of Novel Agents and Strategies into the Management of Soft Tissue Sarcomas

Rashmi Chugh, MD

Professor of Internal Medicine

Sarcoma Clinical Research Program Co-Lead

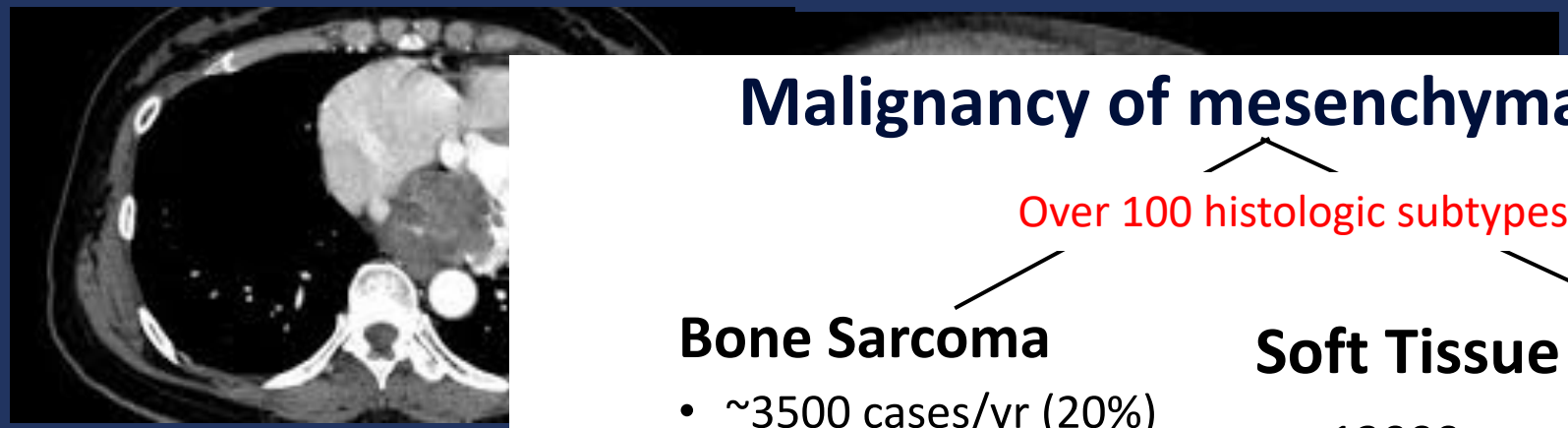
University of Michigan Rogel Cancer Center

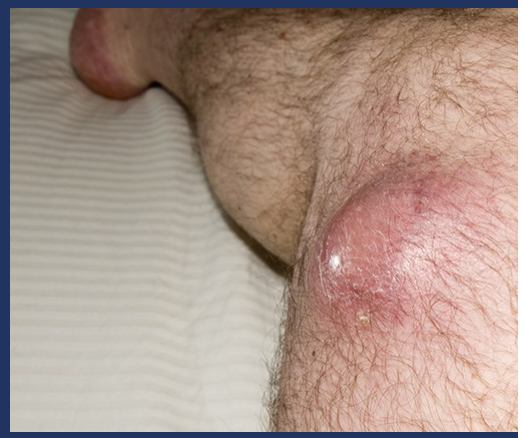
June 3, 2025

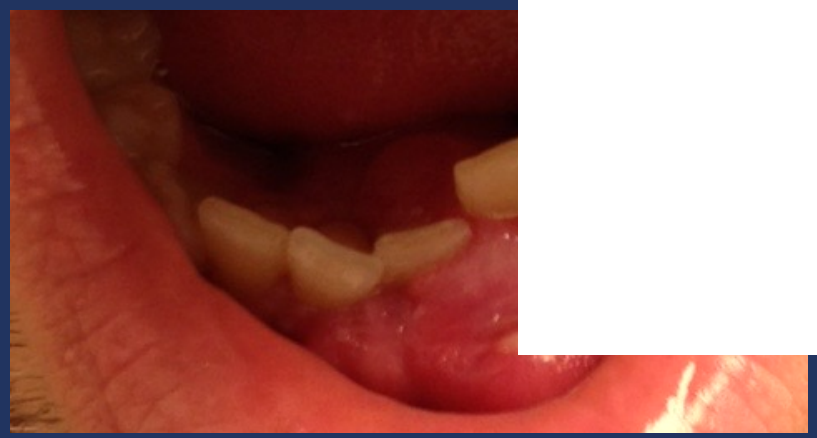
ROGEL CANCER CENTER

Outline

- Introduction and historical approach to advanced soft tissue sarcoma (STS)
- Sequencing treatments and management paradigms for STS
- Novel targeted therapies in specific histologies
  - PEComa
  - Epithelioid Sarcoma
  - Synovial Sarcoma
  - Alveolar Soft Part Sarcoma
- Future Directions







Malignancy of mesenchymal tissue

Over 100 histologic subtypes

Bone Sarcoma

- ~3500 cases/yr (20%)

Major subgroups:

- Osteosarcoma
- Ewing sarcoma
- Chondrosarcoma

Soft Tissue Sarcoma (STS)

- ~13000 cases/yr (80%)
- Major subgroups:
  - Gastrointestinal Stromal Tumor (GIST)
  - Pediatric rhabdomyosarcoma
  - Aggressive “chemo-sensitive” histologies
  - Indolent “chemo-resistant” histologies

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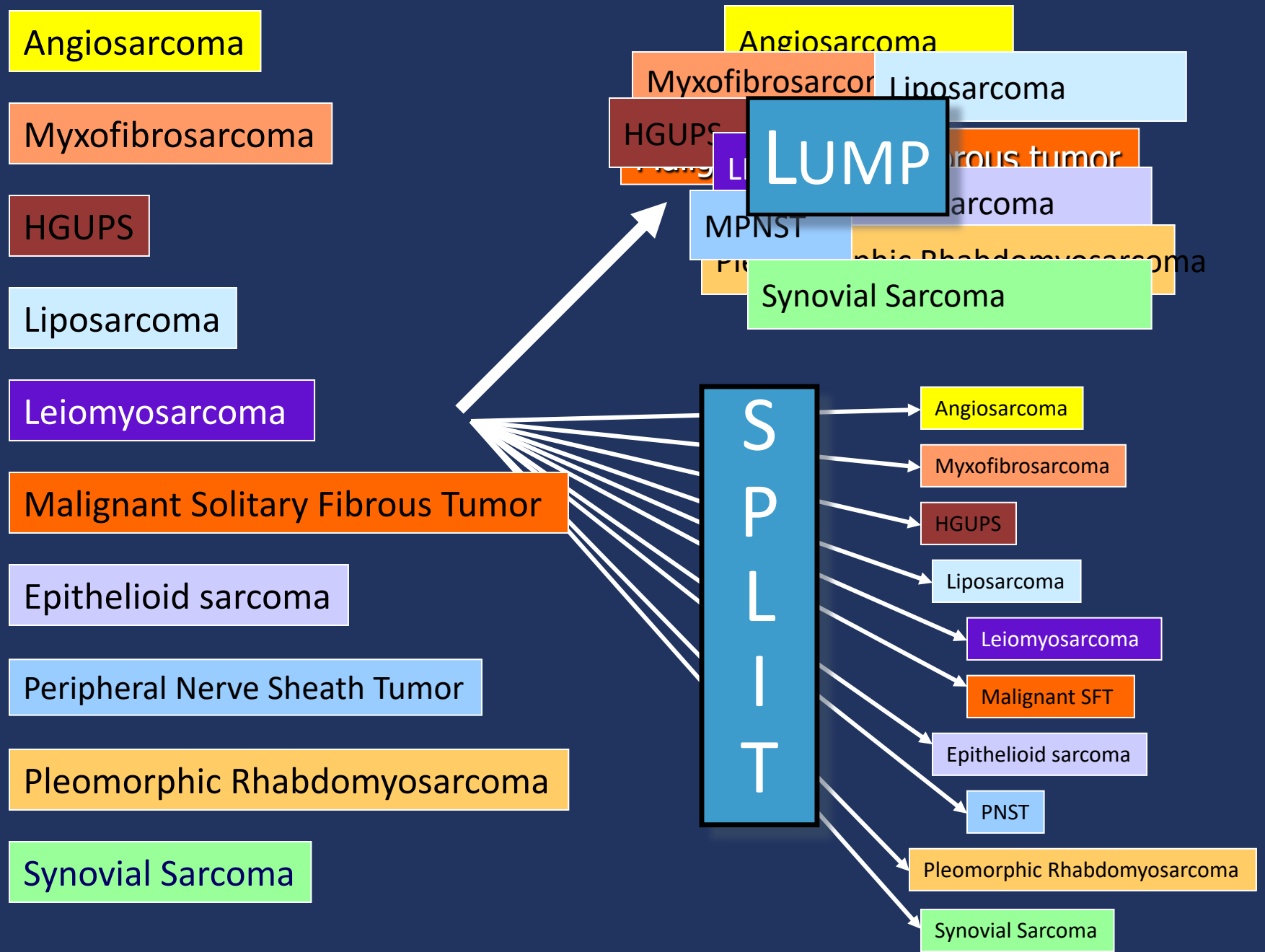

Angiosarcoma
Myxofibrosarcoma
HGUPS
Liposarcoma
Leiomyosarcoma
Malignant Solitary Fibrous Tumor
Epithelioid sarcoma
Peripheral Nerve Sheath Tumor
Pleomorphic Rhabdomyosarcoma
Synovial Sarcoma
LUMP
SPLIT
Angiosarcoma
Myxofibrosarcoma
HGUPS
Liposarcoma
Leiomyosarcoma
Malignant SFT
Epithelioid sarcoma
PNST
Pleomorphic Rhabdomyosarcoma
Synovial Sarcoma

Lumping Therapies: Aggressive STS

Drug	Response rate (%)
Doxorubicin	15% (10-25%)
Ifosfamide	15% (7-41%)
Dacarbazine	10% (4-18%)
Liposomal doxorubicin	10% (9-50%)
Gemcitabine	10% (5-40%)
Pazopanib	4.5%

Common Combinations

Doxorubicin/ifosfamide

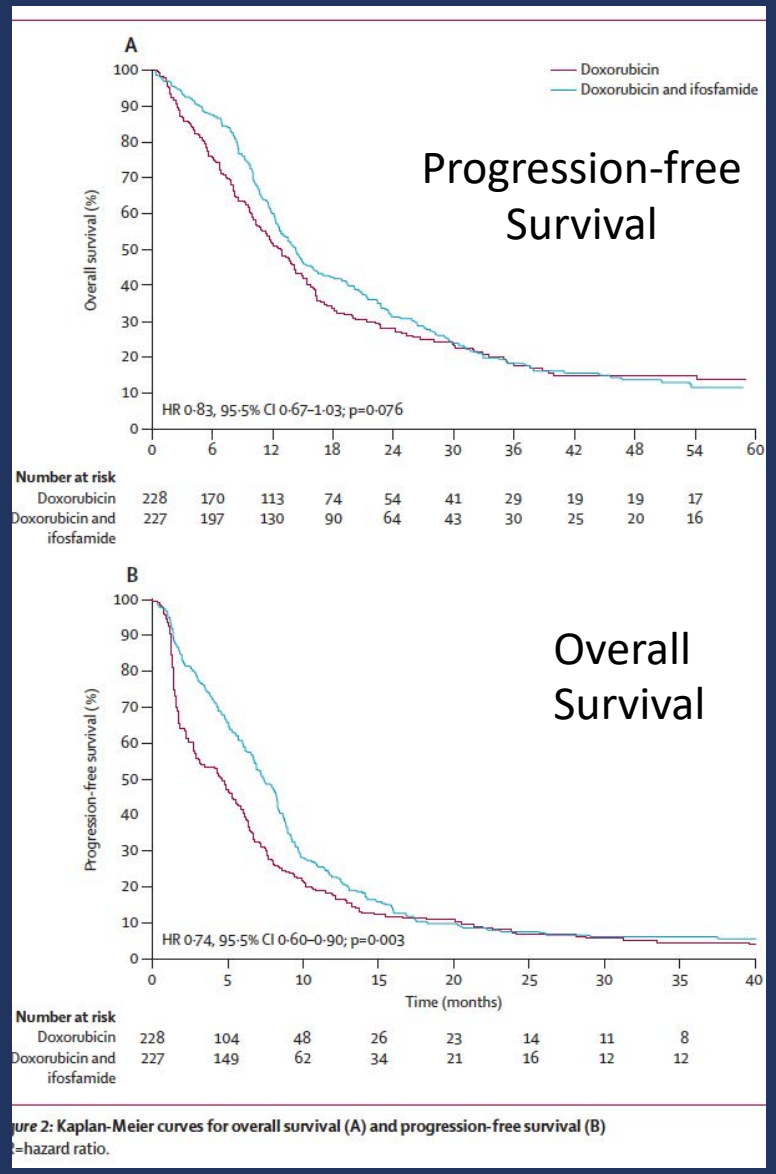
Doxorubicin/dacarbazine

Doxorubicin/trabectedin

Gemcitabine/docetaxel

Gemcitabine/dacarbazine

Therapy for Advanced STS: Single Agent Doxorubicin vs Combination



Progression-free Survival

Overall Survival

ure 2: Kaplan-Meier curves for overall survival (A) and progression-free survival (B)
=hazard ratio.

Doxorubicin alone versus intensified doxorubicin plus ifosfamide for first-line treatment of advanced or metastatic soft-tissue sarcoma: a randomised controlled phase 3 trial

Dox (75 mg/m^2/cycle vs Dox/Ifos (10 g/m^2/cycle)

- No significant difference in median OS: 12.8 vs 14.3 mo (p=0.076)
- Significant difference in median PFS: 4.6 vs 7.4 mo (p=0.003)
- Increased toxicity with combination
  G3/4 Febrile Neutropenia: 13 vs 46%
  Thrombocytopenia: 1 vs 33%
- Increased Response rate with combination: 14% vs 26%

→Reserve doxorubicin combination for fit patients who may benefit from response and can tolerate toxicities

Judson et al; Lancet Oncol. 2014 Apr;15(4):415-23.

Therapy for Advanced STS: Doxorubicin vs Gemcitabine/Docetaxel

Gemcitabine and docetaxel versus doxorubicin as first-line treatment in previously untreated advanced unresectable or metastatic soft-tissue sarcomas (GeDDiS): a randomised controlled phase 3 trial

Dox (75 mg/m^2/cycle vs Gem (675 mg/m2 D1/8)/Doce (75 mg/m^2 D8)

- No significant difference in 24 week-PFS: 46.3% vs 46.4%
- No Significant difference in median PFS: 23.3 vs 23.7 weeks (p=0.06)
- Similar Response Rates: 19% vs 20%
- Similar toxicity with Gem/Doce but overall lower appeal
  - Global health score lower with Gem/doce
  - More infusion trips, more treatment delays, early stopping with gem/doce

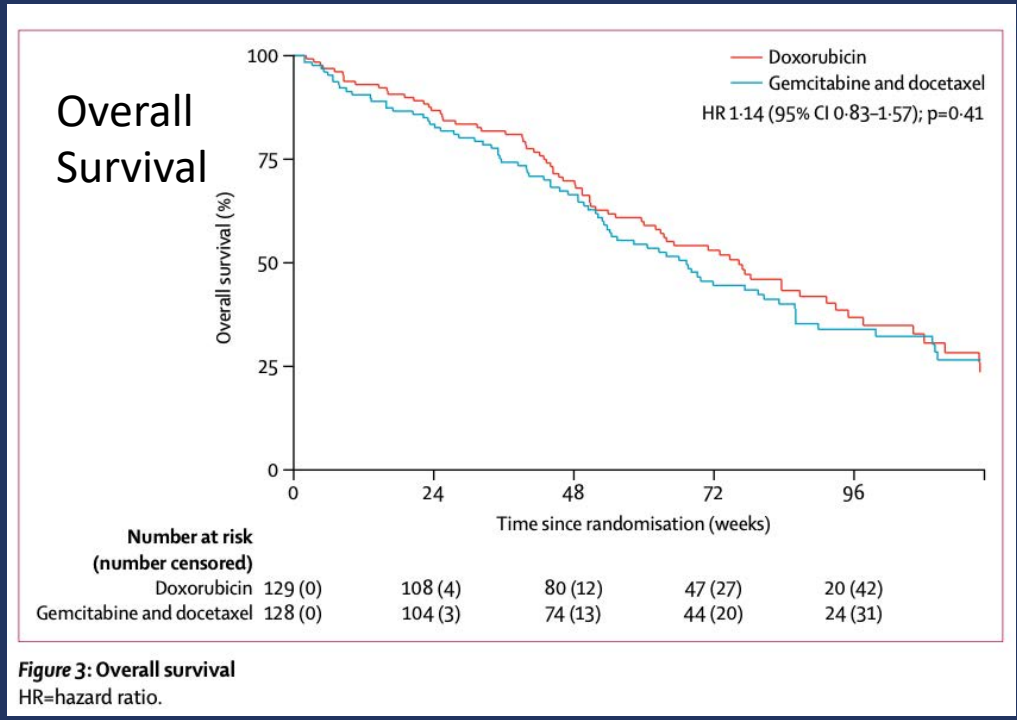


Figure 3: **Overall survival**
HR=hazard ratio.

→Gemcitabine/docetaxel not superior in first line, Doxorubicin should remain as standard of care.

Seddon,et al.The Lancet Oncology, 2017:Volume 18, Issue 10, 1397 - 1410

Approach to Therapy for Advanced STS

- Unlimited spectrum of disease
- Limited patients, across the ages
- Limited effective systemic agents

→ Heavily rely on local therapies even for metastatic disease (surgery, radiation, IR)

- Most histologies start with doxorubicin
- New strategies desperately needed

Molecular Drivers of Sarcoma

Approximately 1/3 of sarcoma with known molecular drivers/aberrations

Sarcoma Histology	Molecular aberration
Gastrointestinal stromal tumor	KIT, PDGFR mutation
Well-differentiated/De-differentiated liposarcoma	12q amplification (MDM2/CDK4)
Leiomyosarcoma	PTEN, Rb, ATRX
PEComa	TSC1/2 mutations

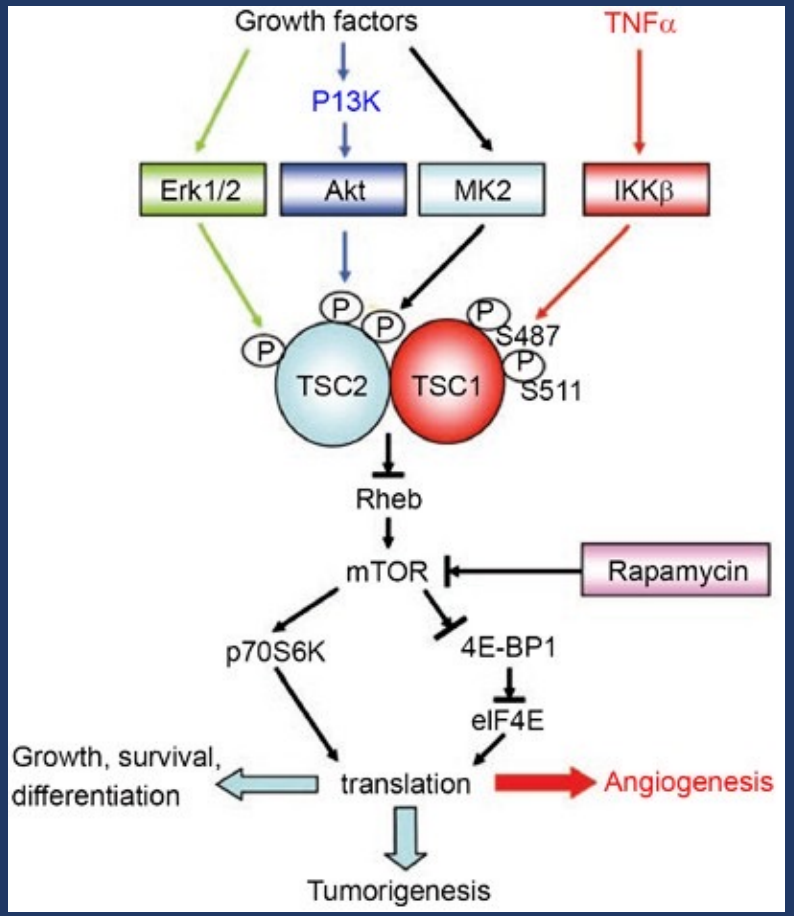
Sarcoma Histology	Translocation
Ewing Sarcoma	EWSR1::FLI1, EWSR1::ERG, FUS::ERG
Synovial Sarcoma	SYT::SSX1, SYT::SSX2, SYT::SSX4
Desmoplastic small round cell tumor	ESWR1::WT1
Alveolar rhabdomyosarcoma	PAX3::FOX01, PAX7::FOX01
Alveolar soft part sarcoma	ASPL::TFE3
Low grade Fibromyxoid Sarcoma	FUS::CREB3L1

→Focus therapies based on molecular driver/histology

Perivascular Epithelioid Cell tumor (PEComa)

- "Ultra-rare" soft tissue sarcoma, often originating in uterus or viscera. Muscle/melanocytic markers
- <~40-80 cases/ year in US
- Molecular aberrations common in TSC1 or TSC2 are common, which negative regulate mTOR signaling pathway. [1.2]
  - Tuberous sclerosis patients predisposed to PEComa
  - Along a spectrum of diseases with lymphangiomyomatosis, angiomyolipoma

- "Malignant" PEComa is aggressive and often metastasizes
  - Cytotoxic chemotherapy with limited benefit
  - mTOR inhibitors have shown variable benefit in case reports[3,4]



FWu, Y., Zhou, B. Kinases meet at TSC. *Cell Res* **17**, 971–973 (2007).

[1]Martignoni et al., Virchows Arch 2008; [2]Gao et al., Signal Transduction 2015 ; [3]Wagner et al., JCO 2010; [4]Dickson et al., Int J Cancer 2013;

nab-Sirolimus

- Albumin-bound sirolimus nanoparticles (ABI-009) - novel IV mTOR inhibitor with significantly higher intratumoral drug accumulation and mTOR target (p70S6k) suppression at equal dose vs oral mTOR inhibitors

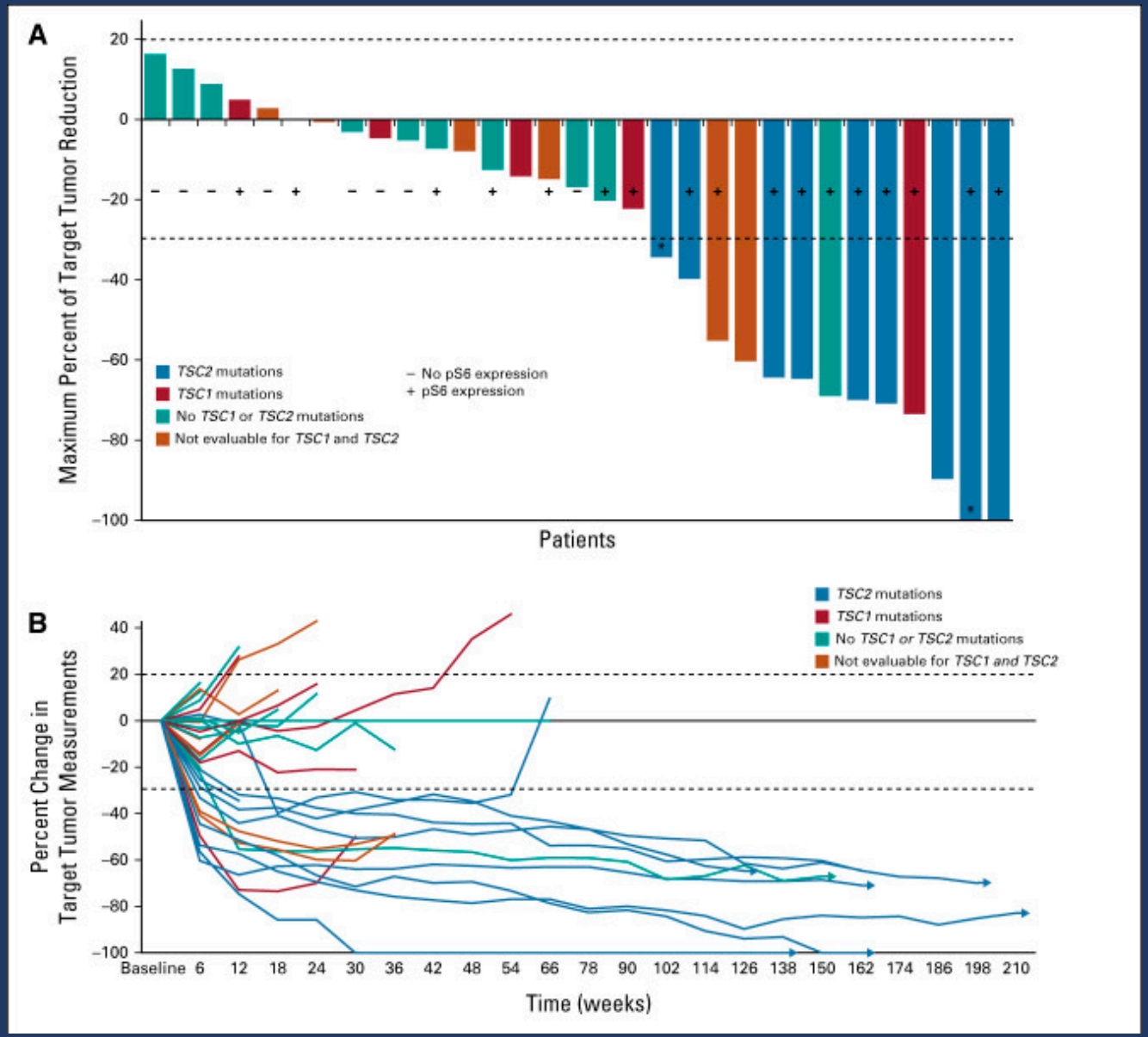
- Oral mTOR inhibitors with variable absorption, require therapeutic level monitoring, and have incomplete target suppression

Phase 2 AMPECT study

- Histologically confirmed advanced PEComa, No prior mTOR Inhibitors

Treatment:

- nab-Sirolimus 100 mg/m^2 IV d 1,8 q 21 d until progression or unacceptable safety



AMPECT: Phase 2 study of nab-Sirolimus in PEComa

31 evaluable patients

Efficacy: ORR 39%, SD 52%
Median PFS 10.6 months
Median OS 40.8 months

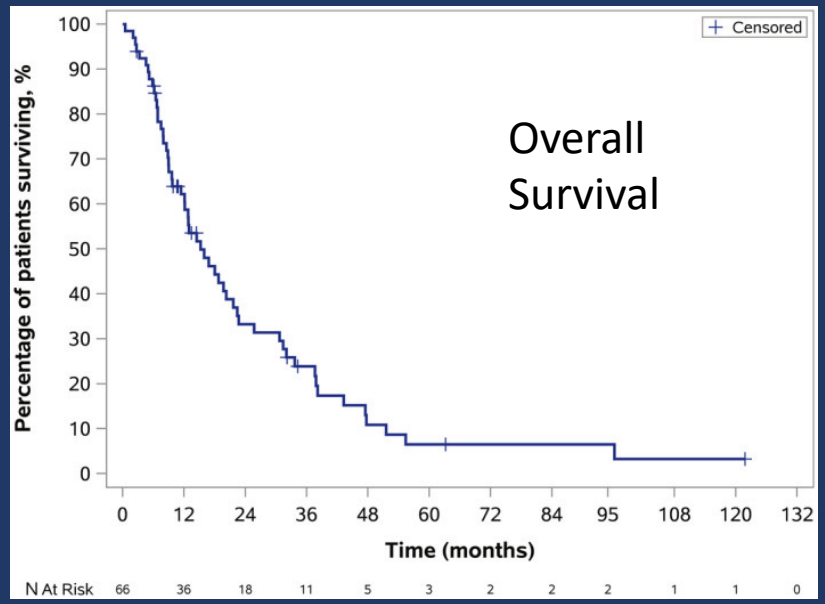
Toxicity:
All grade: Stomatitis (82%), fatigue (62%), Rash (62%)

→ *nab*-sirolimus FDA-approved in 2021 for locally advanced unresectable or metastatic malignant PEComa

Wagner AJ et al. J Clin Oncol. 2024 May 1;42(13):1472-1476

Epithelioid Sarcoma

- Ultra-rare sarcoma, <1% of all STS
- Variable natural history: indolent to aggressive
  - 5 year OS localized disease: 75%
  - 1 year OS metastatic disease: 42%
- Real world data collection from 5 US centers:
  - 74 patients- majority male, median age 33
  - rwORR - 1st line: 14.9%
    2nd line: 9.4%
  - rwDCR - 1st line: 20.3%
    2nd line: 19.8%



- Characterized by loss of INI1/SMARCB1 of the Chromatin remodeling complex causing SWI/SNF dysfunction
- →Leads to aberrant EZH2 activity and oncogenic dependence

Gounder MM, et al. Cancer. 2021 Apr 15;127.

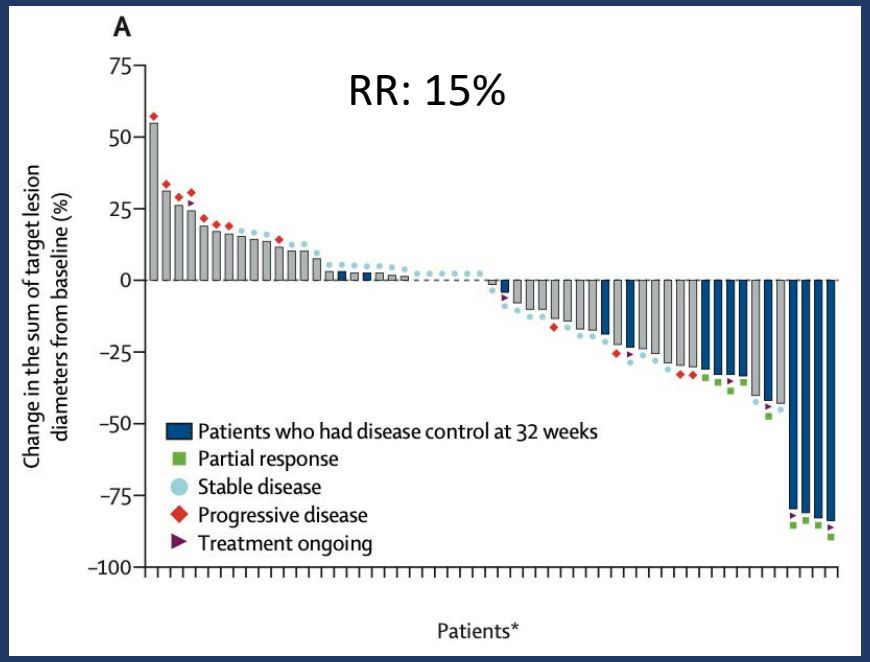
Tazemetostat (EZH2 inhibitor) in advanced epithelioid sarcoma with loss of INI1/SMARCB1

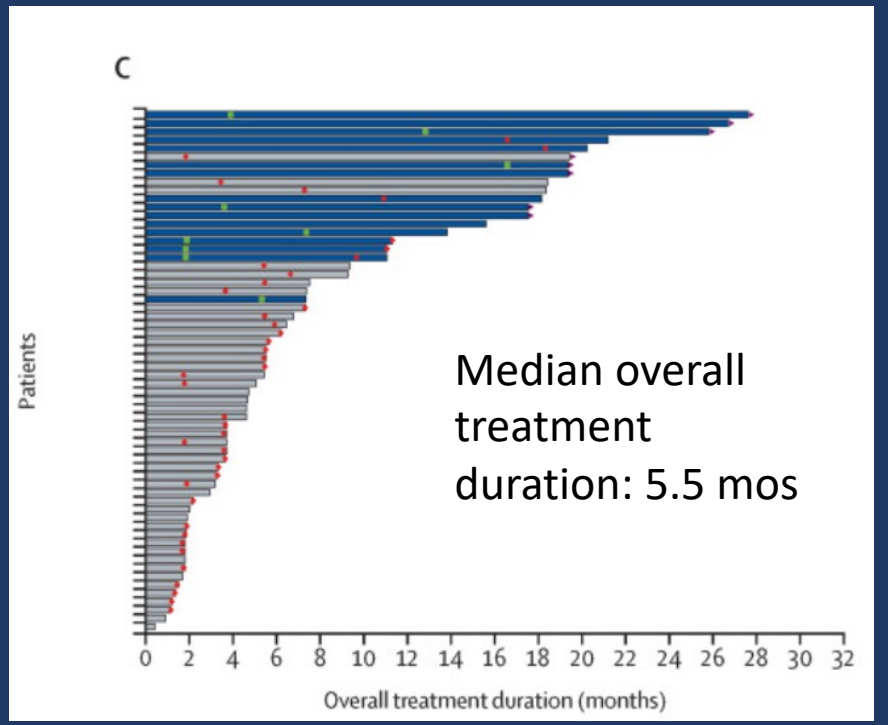
Phase 2 basket study-
Cohort 5 epithelioid sarcoma with confirmed INI-1 loss
Tazemetostat 800 mg twice daily

Efficacy: 62 patients
ORR: 15%, median DOR not reached (9.2mo-NE)
Median PFS 5.5 months
Median OS 19.0 months

Toxicity:
Well tolerated. Grade 3 or worse Aes:
anaemia (four [6%]) and weight loss (two [3%]).

→Tazemetostat received accelerated FDA approval in 2020 for advanced epithelioid sarcoma





Gounder M, et al. Lancet Oncol. 2020 Nov;21(11)

Adoptive T cell therapy in synovial sarcoma

- Synovial sarcoma is a rare aggressive soft tissue sarcoma
  - ~1000 cases annually in US
  - Slight male predominance, younger adult population
- Characterized by SSX-SYT translocation
- Majority of tumors express NY-ESO-1 and MAGE-A4 antigens

Afamitresgene autoleucel (Afami-cel)

- High-affinity TCR therapy to MAGE-A4 antigen
- TCR gene transduced into autologous T cells via lentiviral vector
- Restricted to patients with specific HLA-A*02 genotypes

SPEARHEAD-1 Objectives

SPEARHEAD-1: A Phase 2 trial of afami-cel in patients with advanced synovial sarcoma or MRCLS

KEY ELIGIBILITY CRITERIA

- Advanced synovial sarcoma or MRCLS
- ECOG performance status 0 or 1
- Aged ≥ 16 and < 75 years
- HLA-A*02 positive
- MAGE-A4 expression: ≥ 30% of tumor cells that are ≥ 2+ by immunohistochemistry
- Must have previously received either an anthracycline- or ifosfamide-containing regimen

EFFICACY

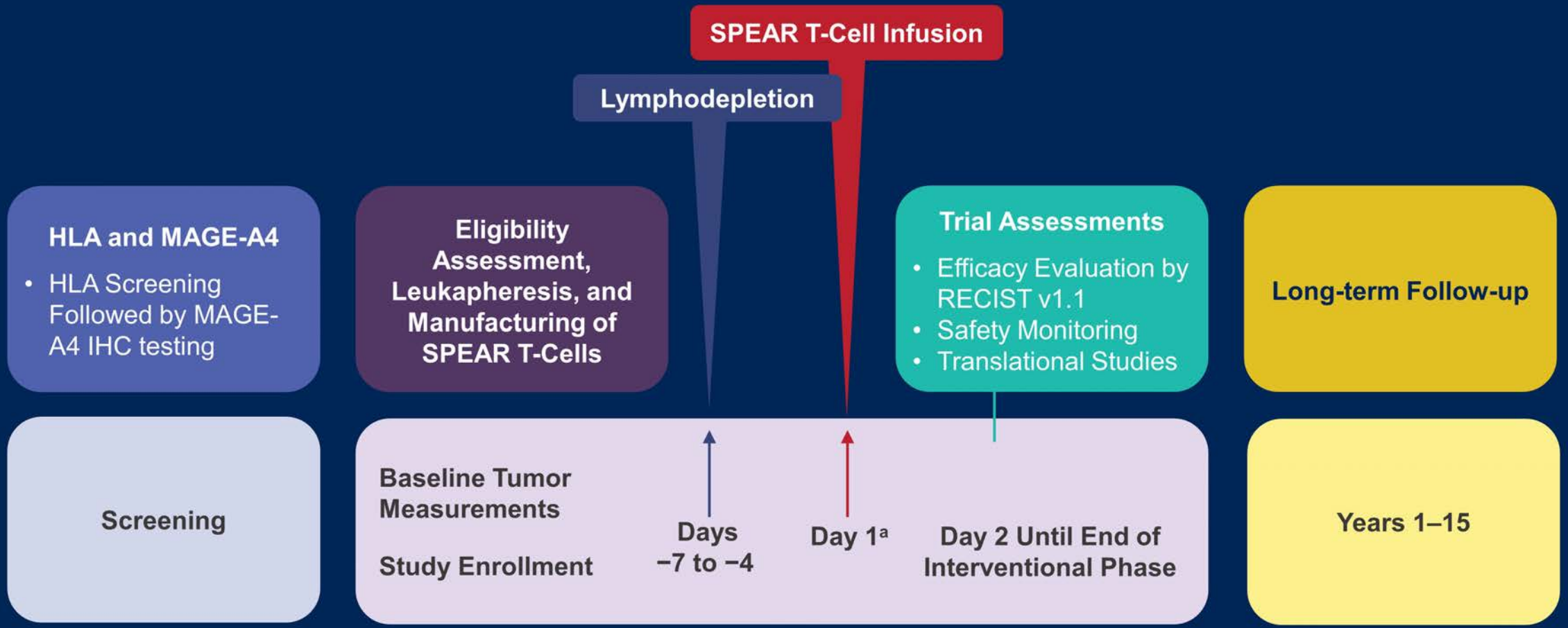
- ORR per RECIST v1.1 by independent review
- Duration of response
- Time to response
- Progression-free and overall survival

SAFETY AND TOLERABILITY

- AEs
- AEs of special interest

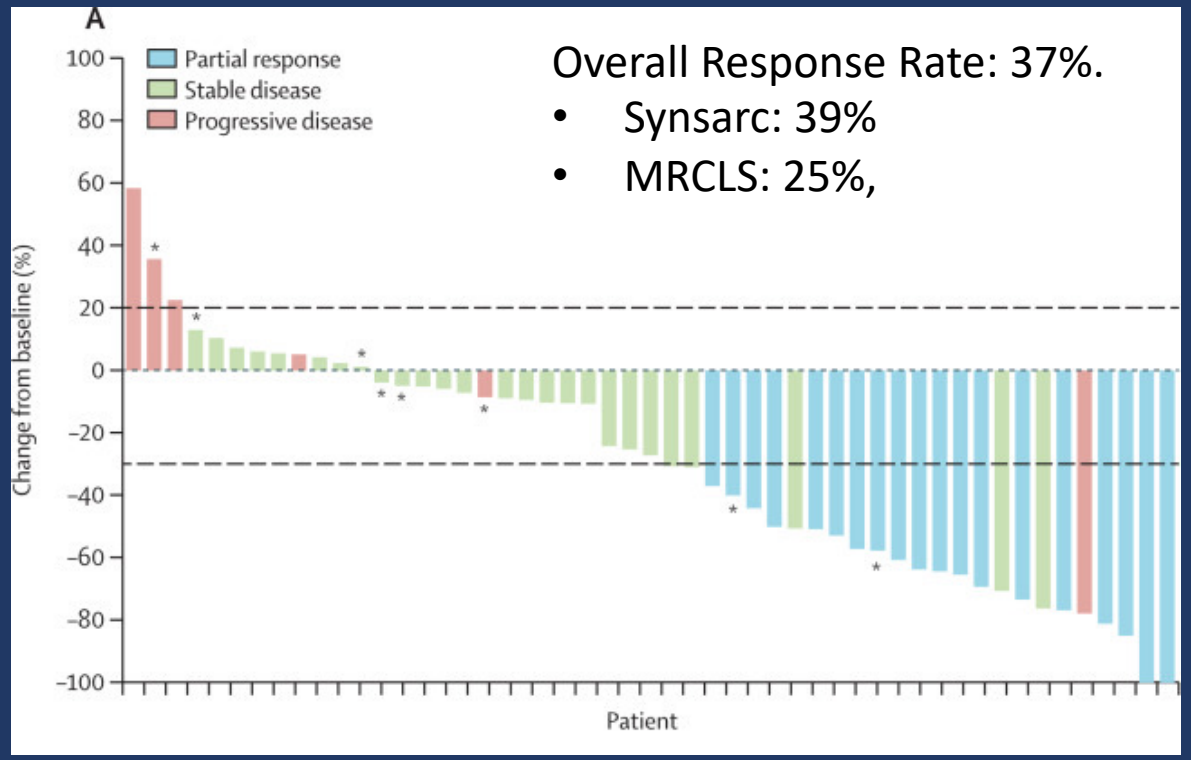
AE = adverse event; ECOG = Eastern Cooperative Oncology Group; MAGE = Melanoma Antigen Gene; MRCLS = myxoid/round cell liposarcoma; ORR = overall response rate; RECIST = Response Evaluation Criteria in Solid Tumours

SPEARHEAD-1 Trial Design



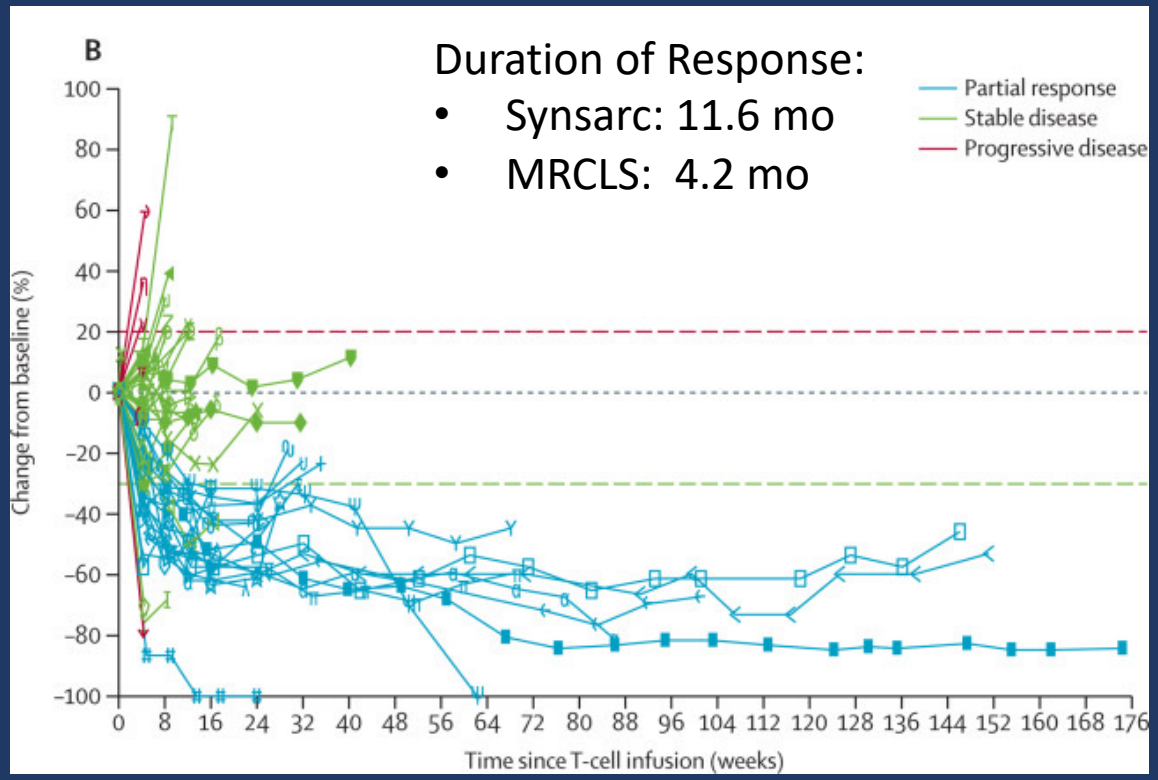
IHC = immunohistochemistry; RECIST = Response Evaluation Criteria in Solid Tumours. [a]Patient is hospitalized for T-cell infusion and discharged at the discretion of the Investigator

SPEARHEAD-1: Afami-cel



Overall Response Rate: 37%.

- Synsarc: 39%
- MRCLS: 25%,



Duration of Response:

- Synsarc: 11.6 mo
- MRCLS: 4.2 mo

Results:
44 patients with synovial sarcoma
8 patients with Myxoid Round Cell Liposarcoma (MRCLS)

AEs:

- Cytokine release syndrome: 71% (1 grade 3 event)
- Grade ≥3 Cytopenias :
  - Lymphopenia 50 [96%]neutropenia 44 [85%],
  - leukopenia 42 [81%]

D'Angelo SP et al. .Lancet. 2024 Apr 13;403(10435):1460-1471.

Synovial Sarcoma Case History

50 y/o presented with dyspnea

→6.7 cm left hilar mass- resected: high grade synovial sarcoma with positive margins. Rx with adjuvant radiation. Recurred L paraspinal.

→Doxorubicin/ifosfamide x 2 cycles with poor tolerance and then single agent doxorubicin →progressive disease

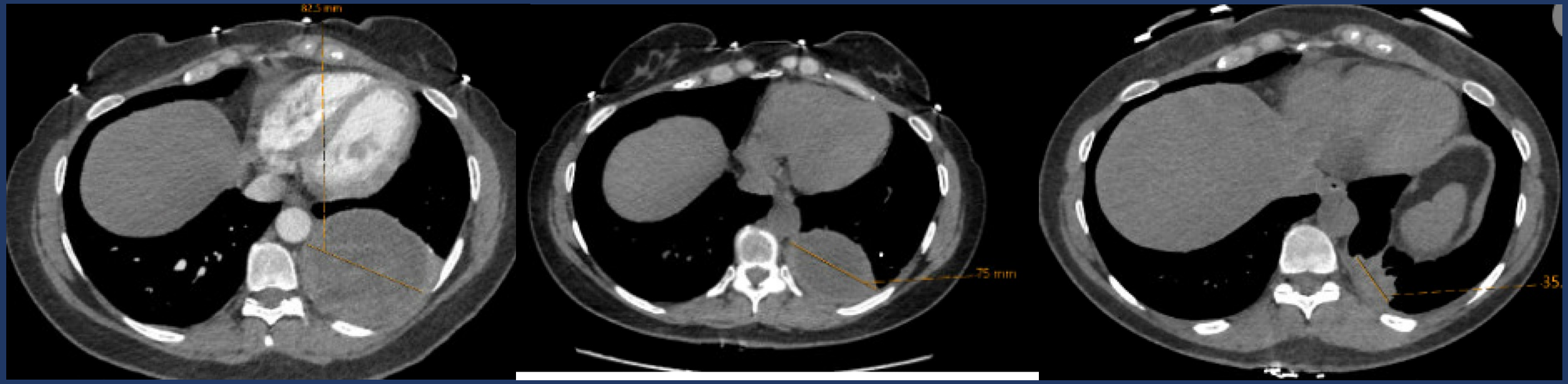
→Pazopanib x 5 months →progressive disease.

→Screened for SPEARHEAD Study- Eligible based on HLA type and MAGEA4 expression. Bridge therapy with trabectedin x 3

→Pheresis

→Fludarabine/Cyclophosphamide conditioning followed by ADP-A2M4 SPEAR T-cells

8/2/24: FDA Approves Afamitrasgene Autoleucel for Metastatic Synovial Sarcoma via Accelerated Approval



Screening scan

4 month scan

12 month scan

Ongoing Partial Response at 12 months!

Only applicable for a minority of pts

Screened (n=330)→HLA-A*02 present (n=176)→MAGE-A4 positive (n=106)→Received T cell therapy (n=37)

Checkpoint inhibitors in STS

Checkpoint inhibitors with greatest signal of activity in few histologies

-Undifferentiated Pleomorphic Sarcoma:

-RR ~14-23%

-Dedifferentiated Liposarcoma:

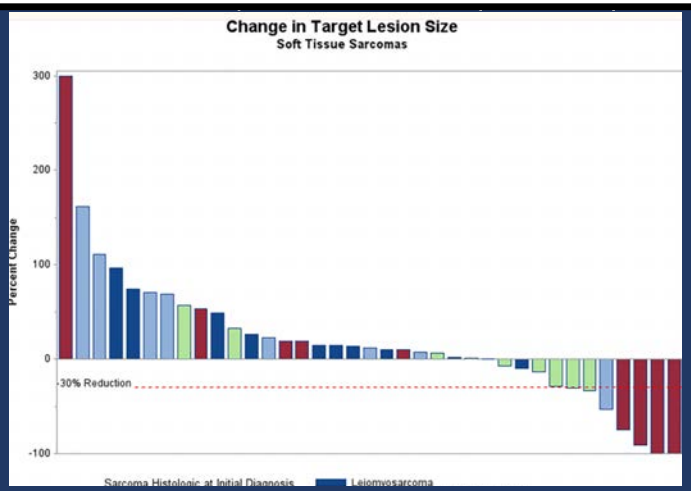
-RR~10%

-Angiosarcoma:

-Nivo/ipi: RR 25% (4/16). 3 CRs in cutaneous. 6-month PFS:38%.[3]

SARC028: Pembrolizumab in STS[1]

Subtype	Responders	RR	Expansion
Leiomyosarcoma	0	0%	
HGUPS	1 CR, 3 PR	40%	23%
Liposarcoma	2 PR	20%	10%
Synovial	1PR	10%	



-PDL1/TMB status not predictive, Class E immune phenotype predictive on correlative studies[2]

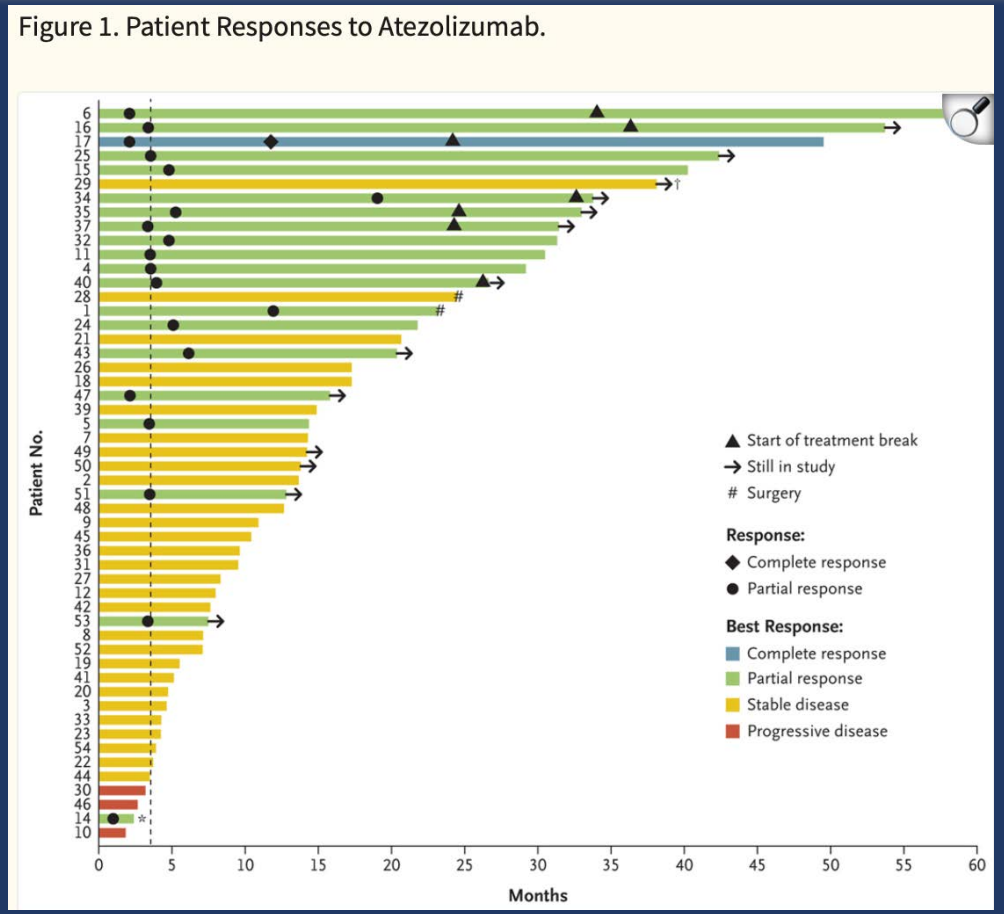
-Positive signals seen in neoadjuvant setting with radiation and UPS/dedifferentiated liposarcoma

[1]Tawbi et al. Lancet Oncol. 2017. [2]Wagner MJ et al. , J Immunother Cancer. 2021. [3]Petitprez F et al. Nature. 2020 Jan

Atezolizumab in Alveolar Soft Part Sarcoma (ASPS)

ASPS is an ultra-rare STS
-most common in AYA, population
-indolent course (5-year OS: 20-46%)
-often metastatic at diagnosis
-ASPL::TFE3
-resistant to cytotoxics

Atezolizumab (PDL1 mAb) Phase 2 study in advanced ASPS
-ORR of 37% (95% CI, 24–51)
-mPFS: 20.8 mos, mDOR: 24.7 mos,



→ *atezolizumab* approved in 2021 for patients ≥2 years old with unresectable or metastatic ASPS

Chen AP, et al. N Engl J Med. 2023 Sep 7;389(10):911-921.

Histology-specific agents in (non-GIST) STS

Histology	FDA approved
Alveolar soft part sarcoma	Atezolizumab
Epithelioid sarcoma	Tazemetostat
Liposarcoma	Trabectedin Eribulin
Leiomyosarcoma	Trabectedin
Synovial sarcoma	Afamitresgene autoleucel
Giant Cell tumor of bone	Denosumab
Desmoid tumor	Nirogacestat
Tenosynovial giant cell tumor	Pexidartinib Vimseltinib

Histology	NCCN Compendia Listed
Angiosarcoma	Paclitaxel, Checkpoint inhibitors
Alveolar soft part sarcoma	Sunitinib, pazopanib, pembrolizumab + axitinib
Undifferentiated pleomorphic sarcoma	Checkpoint inhibitors

Future Directions….Combinations….and more Targeting

Standard +Targeted

- Doxorubicin + Trabectedin (Improved PFS/ORR/OS in LMS, NEJM 2024)
- Doxorubicin +Lurbinectedin in Leiomyosarcoma (NCT06088290)

Immunotherapy ++

- Doxorubicin + pembrolizumab in UPS (NCT06422806)
- Nivolumab + sunitinib (IMMUNOSARC, Asco 2025)
- Tazemetostat + nivolumab + ipilimumab in SMARCA4-deficient (NCT05407441)
- Pembrolizumab +Cabozantinib (PEMBROCABOSARC\NCT05182164)

Antibody-drug Conjugates

- B7H3, KIT

Therapies in STS Summary

- Historically treat most aggressive histologies with doxorubicin initially.
  - Combinations used when applicable
  - Prioritization of quality, toxicity measures in addition to responses
  - Local therapies key adjuncts
  - Many nuances to consider! Refer to sarcoma center when possible
- Recently approved agents are histology and/or pathway specific and are preferred in earlier lines when applicable (.i.e- mTOR, EZH2, afami-cel)
- Future…
  - Combination strategies
  - Many additional pathways of interest based on driver

Thank you!

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ROGEL CANCER CENTER

Agenda

Introduction: Current Role of General Medical Oncologists in the Treatment of Soft Tissue Sarcomas (STS)

Module 1: Incorporation of Novel Agents and Strategies into the Management of STS — Faculty Presentation

Module 2: Incorporation of Novel Agents and Strategies into the Management of STS — Survey Questions

Module 3: Evolving Treatment Paradigm for Locally Aggressive STS — Faculty Presentation

Module 4: Evolving Treatment Paradigm for Locally Aggressive STS — Survey Questions

Module 5: ASCO 2025

RTP RESEARCH TO PRACTICE

Questions from General Medical Oncologists — Overview of STS

- Would need a review from the beginning, I do not see these patients other than a few GIST patients, so I refer them elsewhere in my institution to a sarcoma expert.
- Can you provide a practical overview of the key molecular alterations and typical clinical presentations associated with common STS subtypes, and how these factors influence prognosis and treatment selection?
- Soft tissue sarcomas are so complicated and so rare, that I feel very uncomfortable managing these patients without the assistance of a sarcoma subspecialist.

Questions from General Medical Oncologists — Overview of STS: Diagnosis

- What are key mistakes in the community that providers do not consider when seeing patients in this setting prior to eval at tertiary centers?
- When sarcoma is suspected by imaging, do you recommend core needle biopsy or referral to specialty surgeon first?

Questions from General Medical Oncologists — Overview of STS: Molecular Testing

- What molecular testing should be done for a patient with sarcoma?
- How to select therapy based on histology
- I would like to know more about molecular alterations and its implications on prognosis and treatment

Questions from General Medical Oncologists — Perivascular Epithelioid Cell tumor (PEComa)

- How strong is the clinical evidence supporting mTOR inhibitors in PEComa, and what are the key clinical features that would prompt you to consider this therapy?
- Use of nab-sirolimus? No experience
- Have you used nab-sirolimus as neoadjuvant therapy in borderline resectable perivascular epithelioid cell tumors (PEComas)?

Questions from General Medical Oncologists — PEComa

- 51-year-old man was found to have 4 cm right breast mass. Pathology showed PEComa. Any role for adjuvant therapy?
- What therapy do you give after progression on nab-sirolimus?

Agenda

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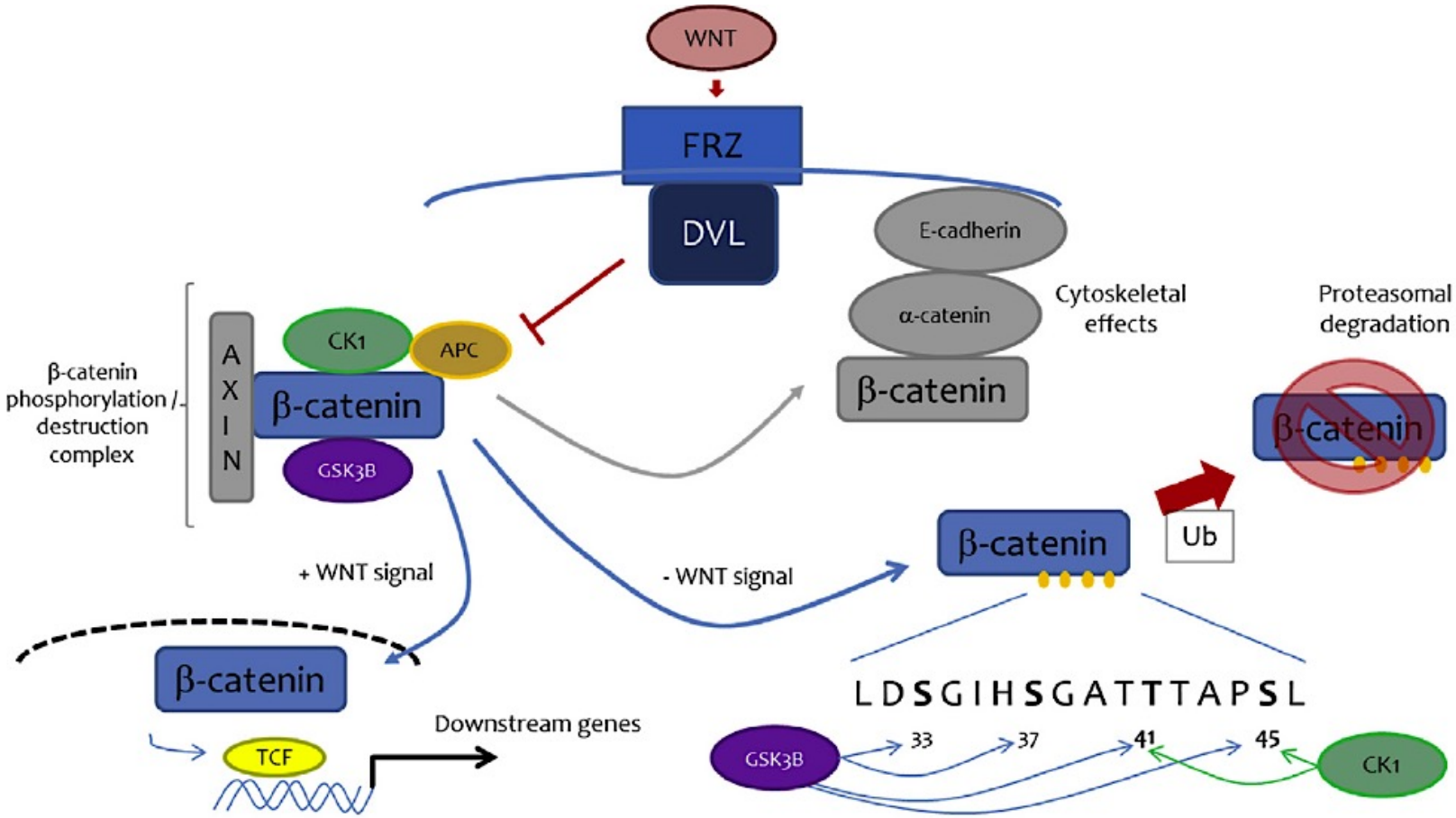
RTP RESEARCH TO PRACTICE

Desmoid Tumor and TGCT

Mrinal Gounder, MD
Sarcoma Medical Oncology | Phase 1 Drug Development Program
Physician Ambassador – India and Asia
Memorial Sloan Kettering Cancer Center

email – gounderm@mskcc.org

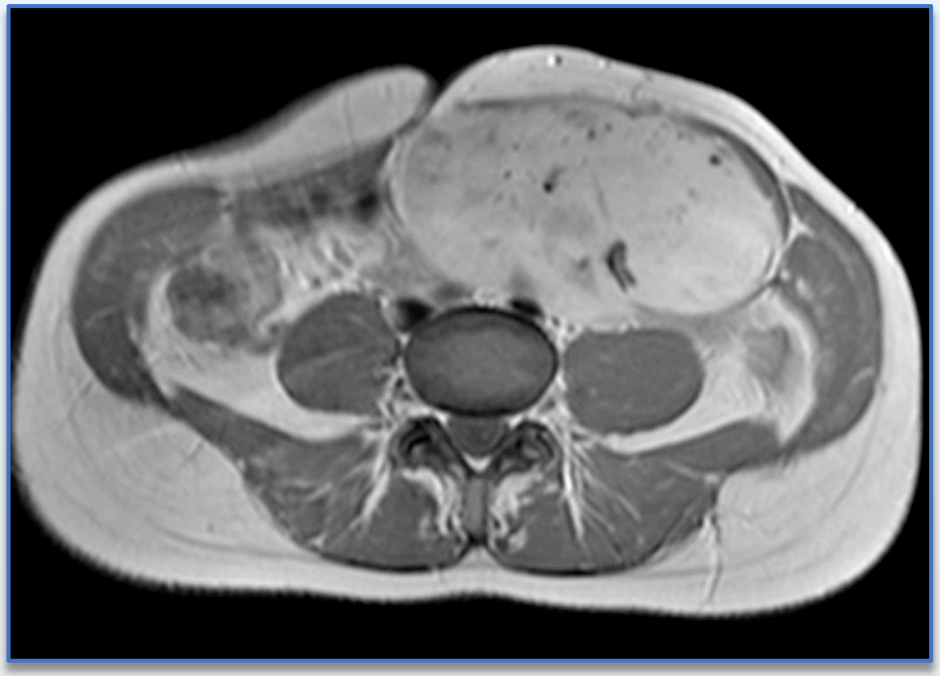
Either *APC* loss or *CTNNB1 (beta-catenin)* mutation found in DT



- Both are mediators of the wingless signaling pathway, which gives rise to an uncontrolled proliferation of fibroblasts

Desmoid Tumor Working Group. Eur J Cancer. 2020 Mar:127:96-107.

Criteria for Active Treatment



- ✓ Cosmesis
- ✓ Pregnancy
- ✓ Progression
- ✓ Pain

Surgery remains in the initial lines of treatments

Tyrosine Kinase Inhibitors

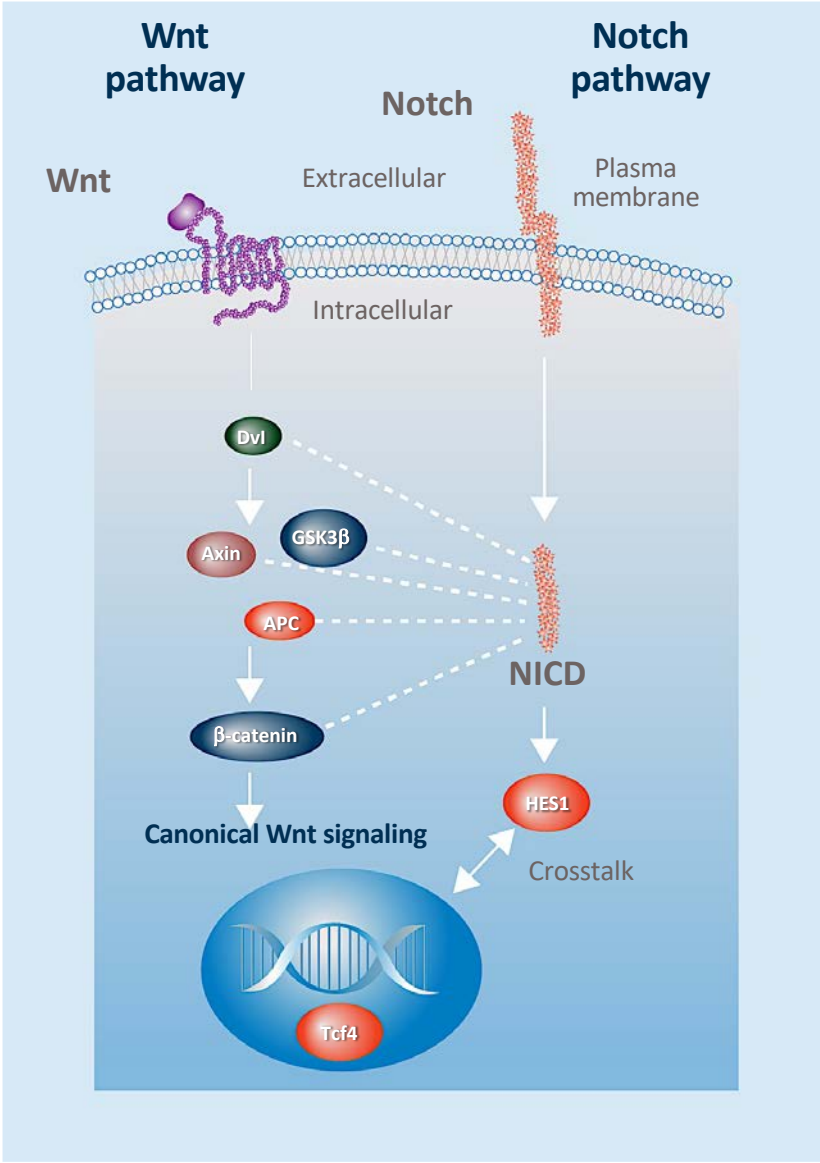
Reference	n	Inclusion Criteria	Treatment Dose, mg	Treatment Duration	ORR, %	6-Month PFS, %	12-Month PFS, %	24-Month PFS, %
Heinrich et al, 2006	19	"Heavily pretreated patients"	Imatinib 800 mg	325 days	16	53	37	NE
Penel et al, 2010	35	"Radiological evidence for PD"	Imatinib 400 mg	1 year	11	80	67	55
Chugh et al, 2010	49	"Locally advanced disease"	Imatinib 200-600 mg	Until PD 9 patients >3 years	6	84	66	NE
Kasper et al, 2017	38	RECIST PD	Imatinib 800 mg	2 years	19	65	59	45
Sorafenib Gounder, 2018	50	"Progressive or symptomatic"	Sorafenib 400 mg	Until PD	33	NE	89	81
Pazopanib Toulmond, ASCO 2018	48	RECIST PD	Pazopanib 800 mg	1 year	37	81	86	NE
Nirogacestat Kummar, 2017	17	"Progressive/ symptomatic"	Nirogacestat 300 mg	Until PD	29	100	100	100

Gamma Secretase Inhibition in Desmoid Tumors

- There is mechanistic rationale for the use of GSIs in DTs because these tumors highly express Notch, which can be blocked by GSIs[5,6]

Nirogacestat is an investigational, oral, selective, small-molecule GSI that has shown evidence of antitumor activity in DTs in phase 1 and 2 trials with a manageable AE profile[1,7,8]

AL102 is an investigational, oral, potent inhibitor of gamma secretase

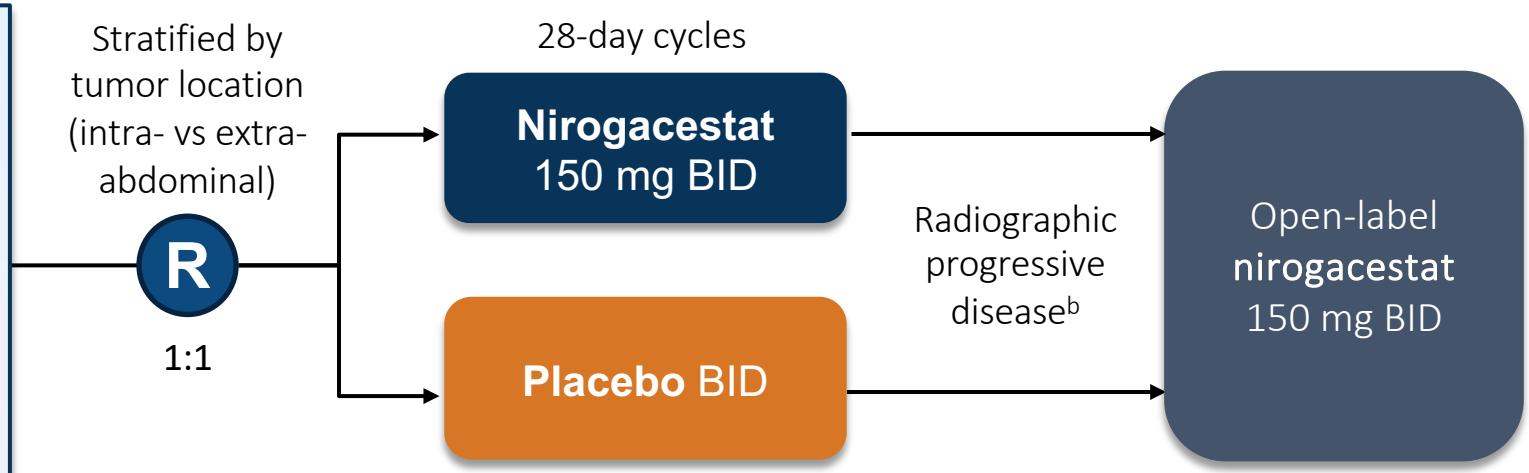


DeFi: Phase 3 Study of Nirogacestat vs Placebo

- Global, randomized, double-blind, placebo-controlled, phase 3 trial comparing the efficacy, safety, and tolerability of nirogacestat vs placebo in adult patients with progressing DTs
- 142 patients randomized across 37 sites in North America and Europe

Adult eligible patients

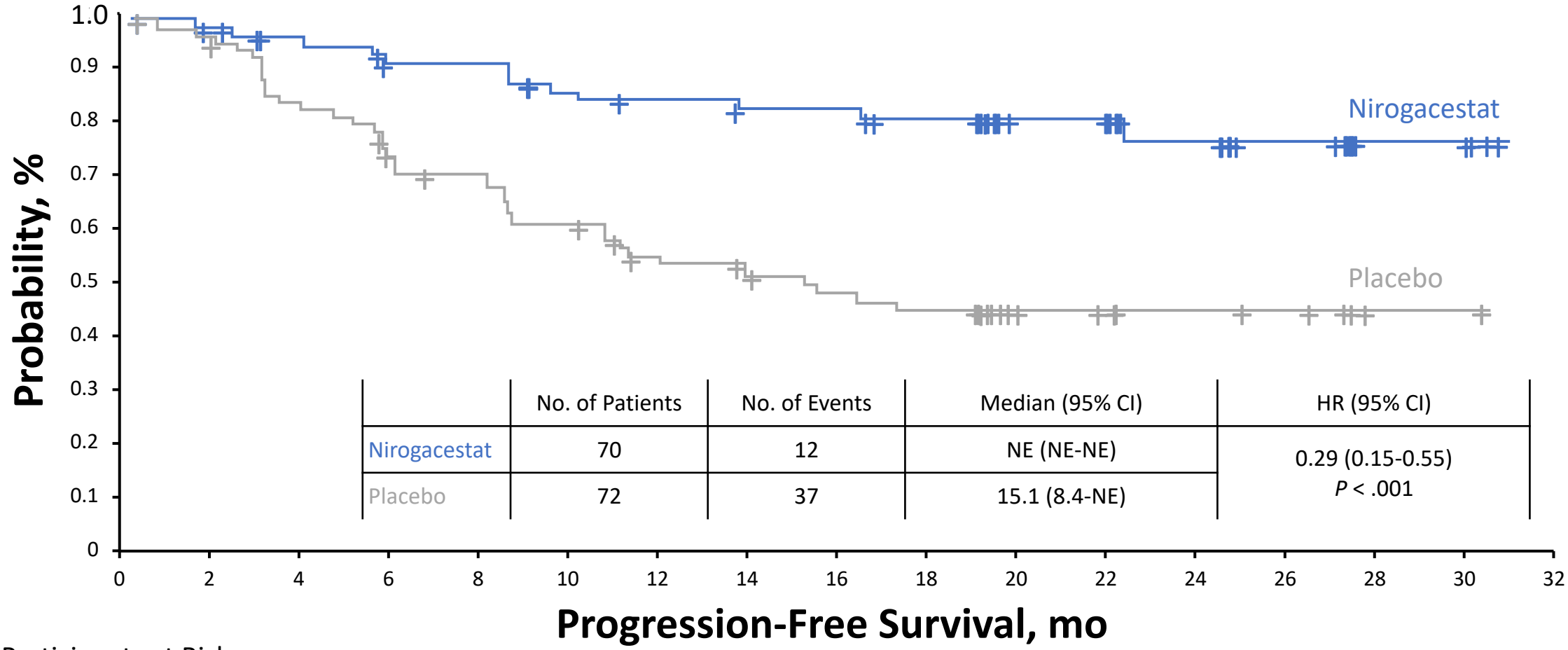
- Histologically confirmed DT with progressive disease per RECIST v1.1[a]
  - Treatment-naive with DT not amenable to surgery, *or*
  - Refractory or recurrent disease (after ≥1 line of therapy)



Primary Analysis Data Cutoff: April 7, 2022

- **Primary endpoint:** PFS[c]
- **Secondary endpoints:** ORR and PROs, including symptom burden, physical/role function, and overall QoL[d]

Nirogacestat Significantly Reduced the Risk of Disease Progression



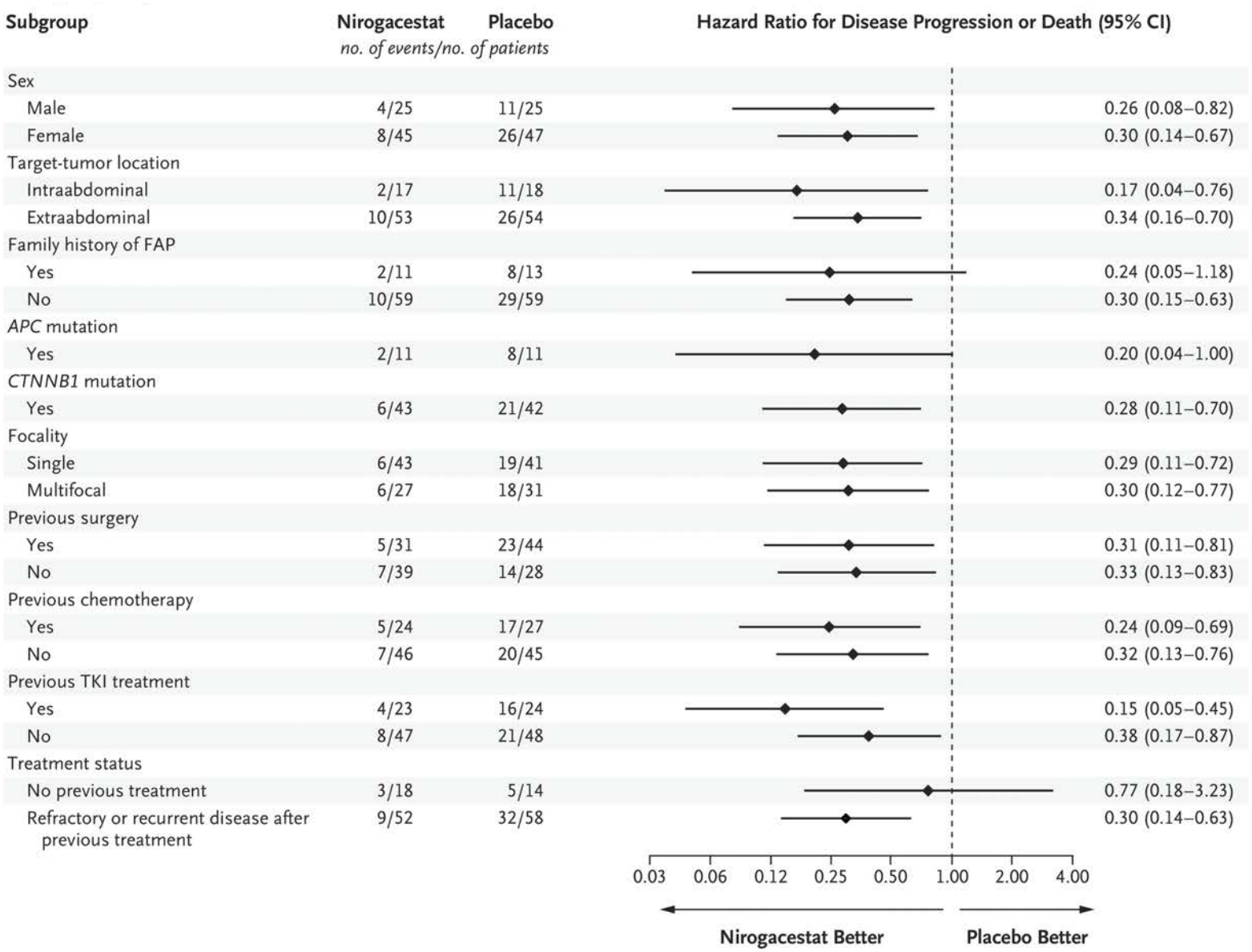
	No. of Patients	No. of Events	Median (95% CI)	HR (95% CI)
Nirogacestat	70	12	NE (NE-NE)	0.29 (0.15-0.55) *P* < .001
Placebo	72	37	15.1 (8.4-NE)	

No. of Participants at Risk

Nirogacestat	70	63	56	52	52	47	46	44	44	41	26	26	17	12	4	4	0
Placebo	72	67	58	47	45	40	32	29	27	25	10	8	6	5	1	1	0

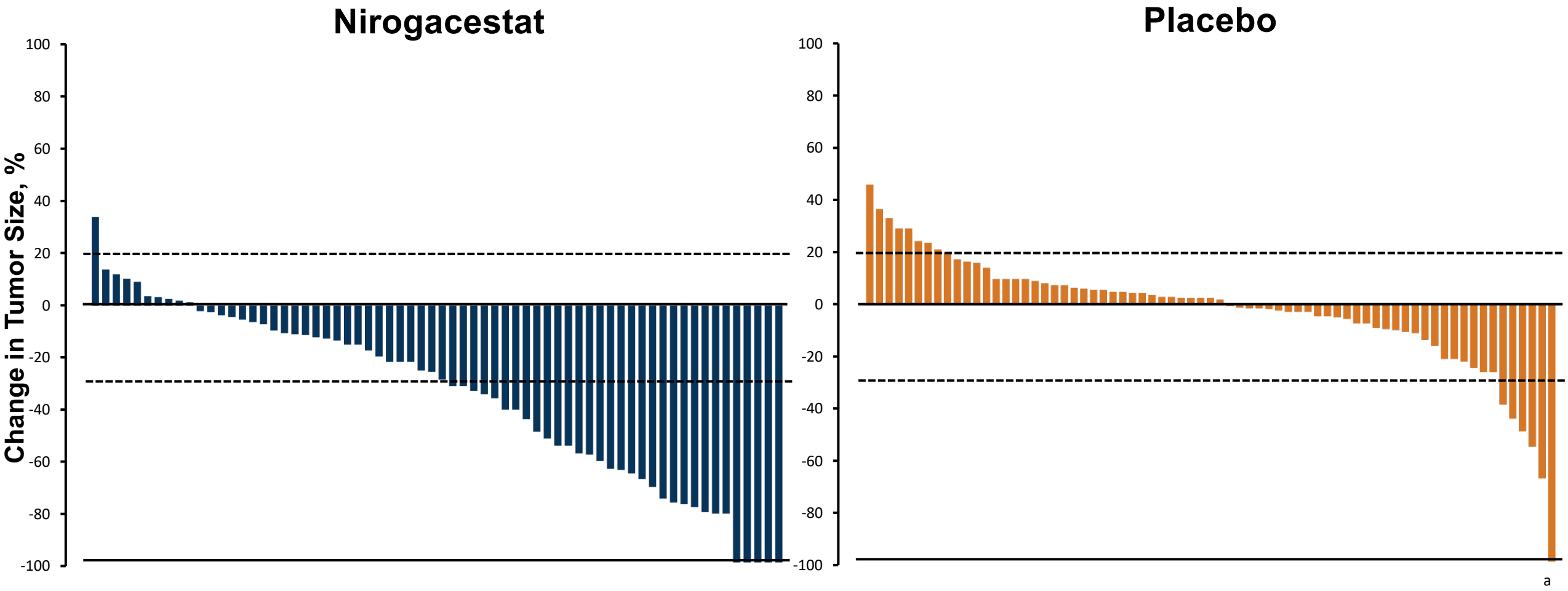
Gounder M et al. NEJM 2023

PFS Benefit With Nirogacestat Was Observed Across Pre-specified Subgroups



Gounder M et al. NEJM 2023

Nirogacestat Resulted in Substantial Reductions in Tumor Size

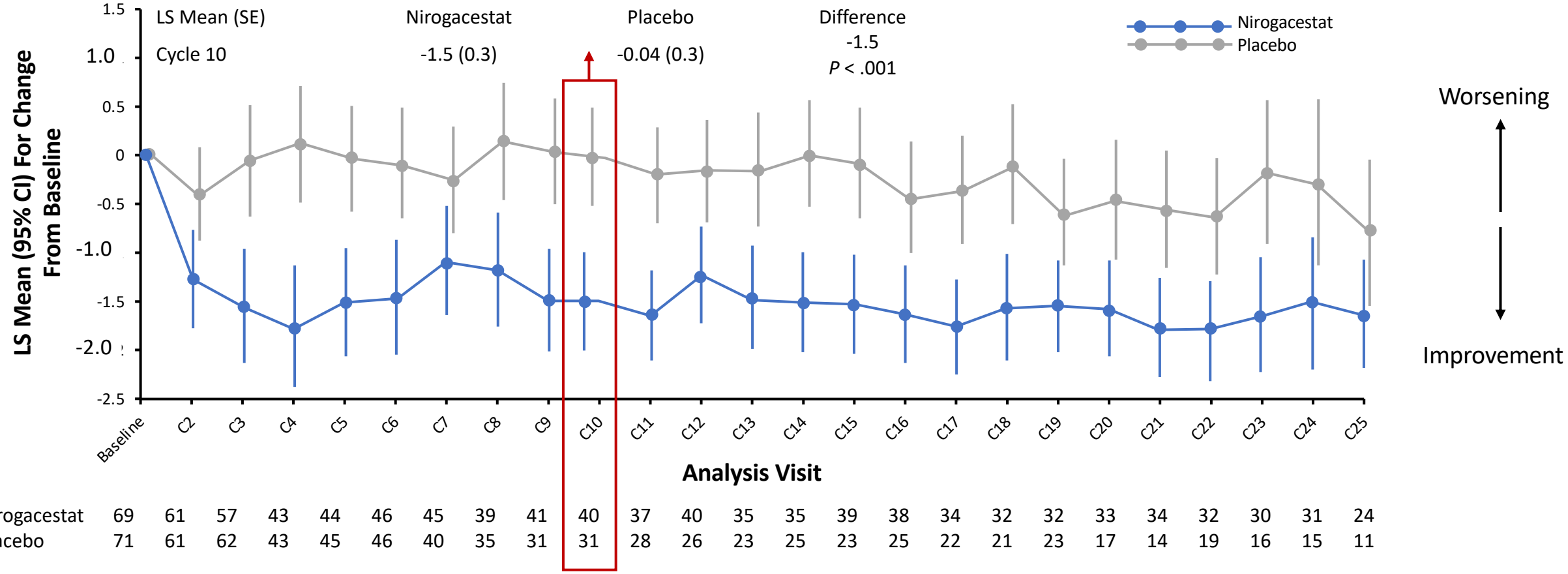


[a] Patient had a complete resolution of the target lesion but still had documented non-target lesion; therefore, not a complete response. Best percent change values are averaged between 2 blinded independent reviewers unless a reader was selected for adjudication, in which case only the adjudicated value is presented.

1. Kasper B et al. ESMO 2022. Abstract LBA2.

Nirogacestat Significantly Reduced Pain

Brief Pain Inventory Short Form: Worst Pain Intensity

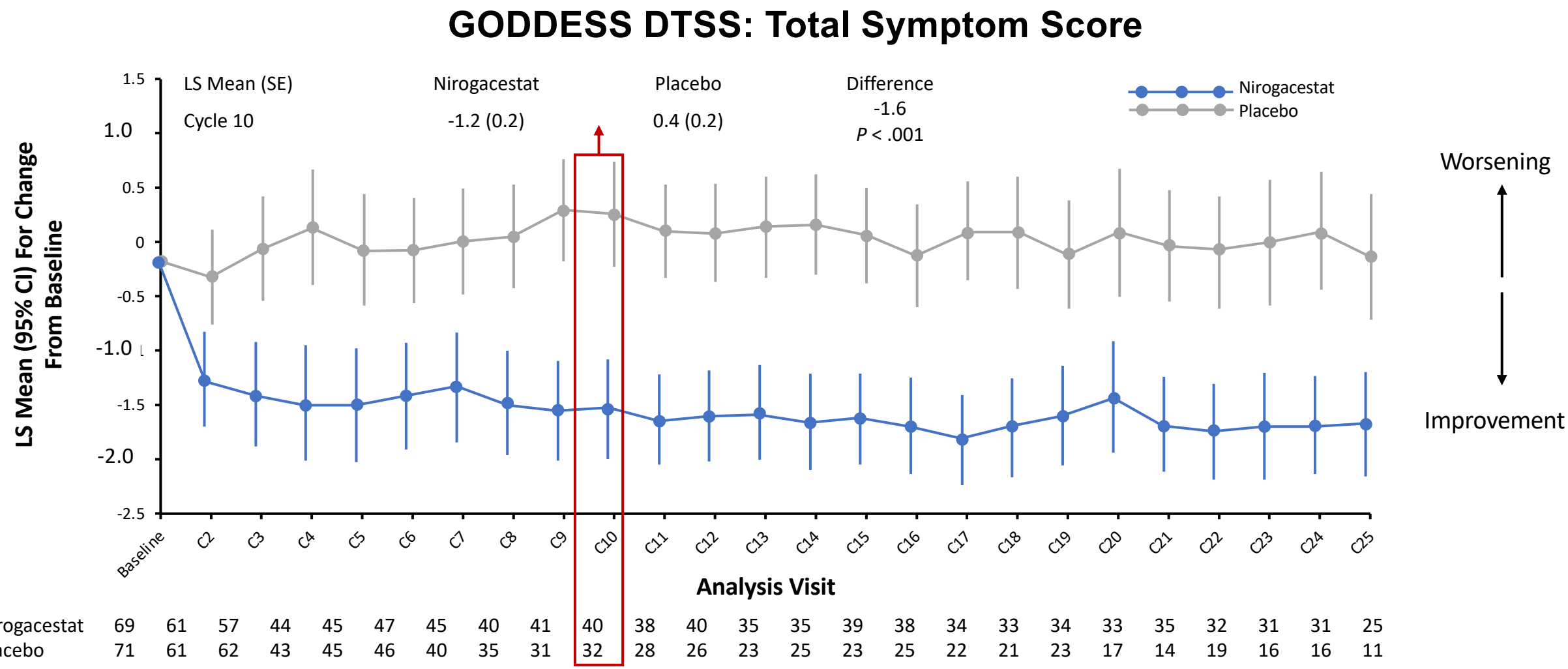


Nirogacestat	69	61	57	43	44	46	45	39	41	40	37	40	35	35	39	38	34	32	32	33	34	32	30	31	24
Placebo	71	61	62	43	45	46	40	35	31	31	28	26	23	25	23	25	22	21	23	17	14	19	16	15	11

Mean (SD) baseline scores: nirogacestat, 3.2 (3.26); placebo, 3.3 (3.31). Differences at cycle 10 were statistically significant and clinically meaningful. LS mean change from baseline represents the 7-day average of "worst pain in last 24 hours".

1. Kasper B et al. ESMO 2022. Abstract LBA2.

Nirogacestat Significantly Reduced DT-Specific Symptom Severity



Mean (SD) baseline scores: nirogacestat, 3.4 (2.34); placebo, 3.5 (2.57). Differences at cycle 10 were statistically significant and clinically meaningful. DTSS total symptom score includes pain, fatigue, swelling, muscle weakness, and difficulty moving.

1. Kasper B et al. ESMO 2022. Abstract LBA2.

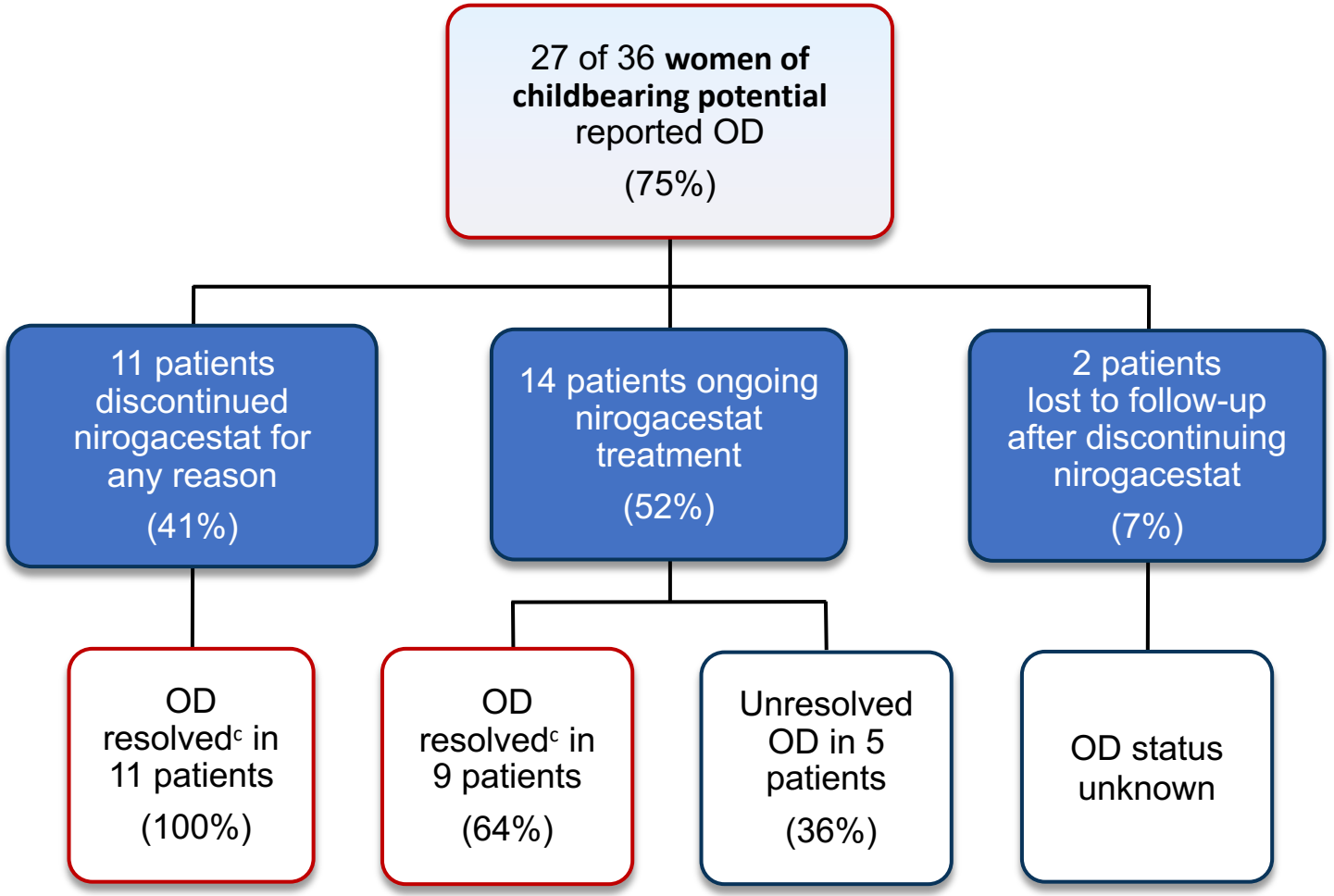
Nirogacestat Safety Profile

Safety Population, n (%)	Nirogacestat (n = 69)		Placebo (n = 72)	
Duration of study drug exposure, median (range), mo	20.6 (0.3-33.6)		11.4 (0.2-32.5)	
Dose intensity, median (range), mg/d	288.3 (169-300)		300.0 (239-300)	
	Any Grade	**Grade ≥3**	**Any Grade**	**Grade ≥3**
Any TEAE	69 (100)	39 (57)	69 (96)	12 (17)
TEAEs of any grade reported in ≥25% of patients in either arm				
Diarrhea	58 (84)	11 (16)	25 (35)	1 (1)
Nausea	37 (54)	1 (1)	28 (39)	0
Fatigue	35 (51)	2 (3)	26 (36)	0
Hypophosphatemia	29 (42)	2 (3)	5 (7)	0
Rash, maculopapular	22 (32)	4 (6)	4 (6)	0
Headache	20 (29)	0	11 (15)	0
Stomatitis	20 (29)	3 (4)	3 (4)	0
TEAEs leading to death	0		1 (1)[a]	
Dose reductions due to TEAEs	29 (42)		0	
Discontinuations due to TEAEs	14 (20)[b]		1 (1)[b]	

- 95% of TEAEs were grade 1 or 2; the first onset of TEAEs in most patients occurred during cycle 1

Frequency and Resolution of Ovarian Dysfunction Observed With Nirogacestat

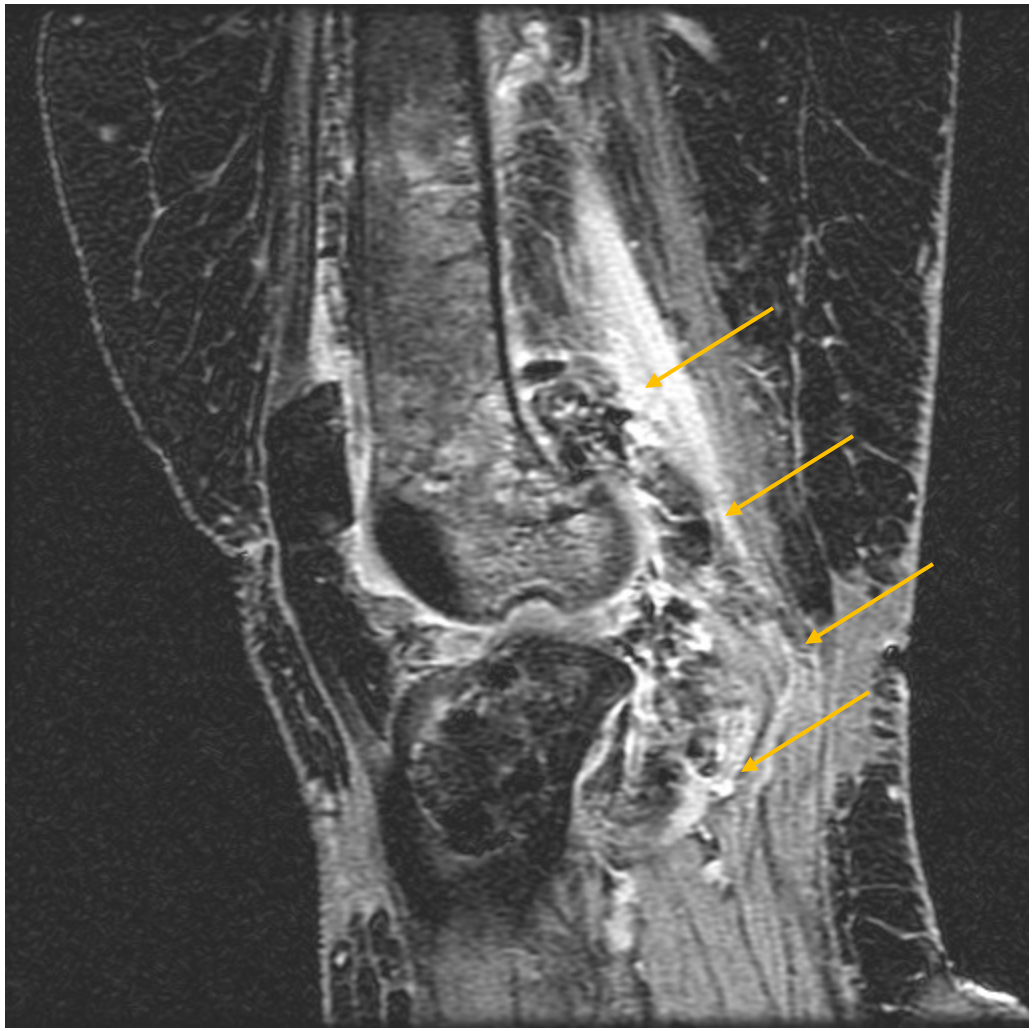
- OD is a composite AE associated with changes in female reproductive hormone levels and clinical manifestations[2,3]
- Protocol-mandated serum hormone collection at baseline and cycles 1, 2, 4, and every 3 thereafter
- Among women of childbearing potential, OD was observed in 75% receiving nirogacestat and 0% receiving placebo
  - Median time to first onset of OD: 8.9 weeks
  - Median duration of OD events: 21.3 weeks



OD, ovarian dysfunction

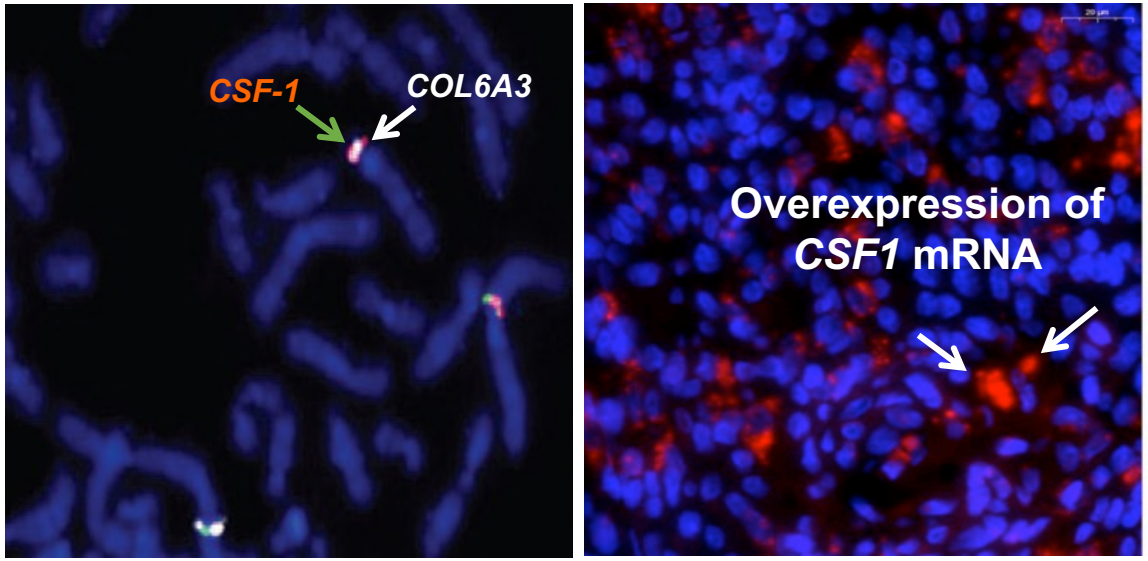
1. Thurston et al. Obstet Gynecol Clin North Am. 2011;38:489-501.
2. Mauri et al. Front Endocrinol (Lausanne). 2020;11:572388.
3. Kasper B et al. ESMO 2022. Abstract LBA2.

Diffuse-Type Tenosynovial Giant Cell Tumor (Pigmented Villonodular Synovitis)

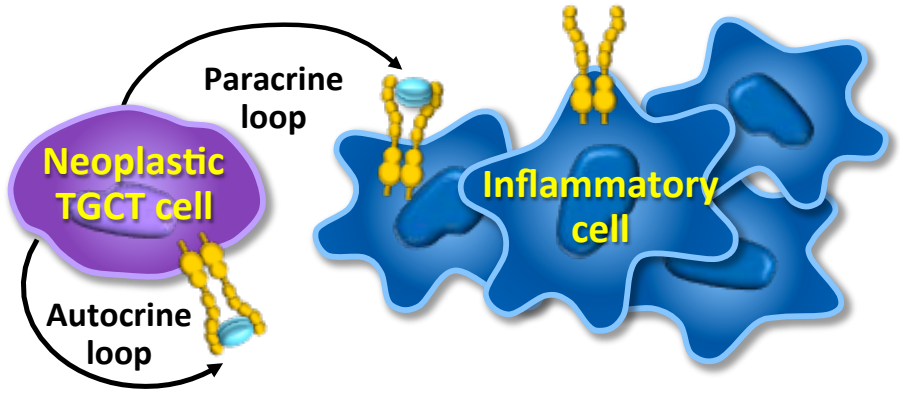


Pathogenesis of TGCT and Rationale for CSF1 Targeting

CSF1/COL6A3 translocation causes CSF-1 overexpression in a subpopulation of neoplastic cells, resulting in the recruitment of CSF-1 receptor-bearing inflammatory cells, leading to tumor formation[1]



The tumor primarily consists of mononuclear and multinucleated giant cells[1]



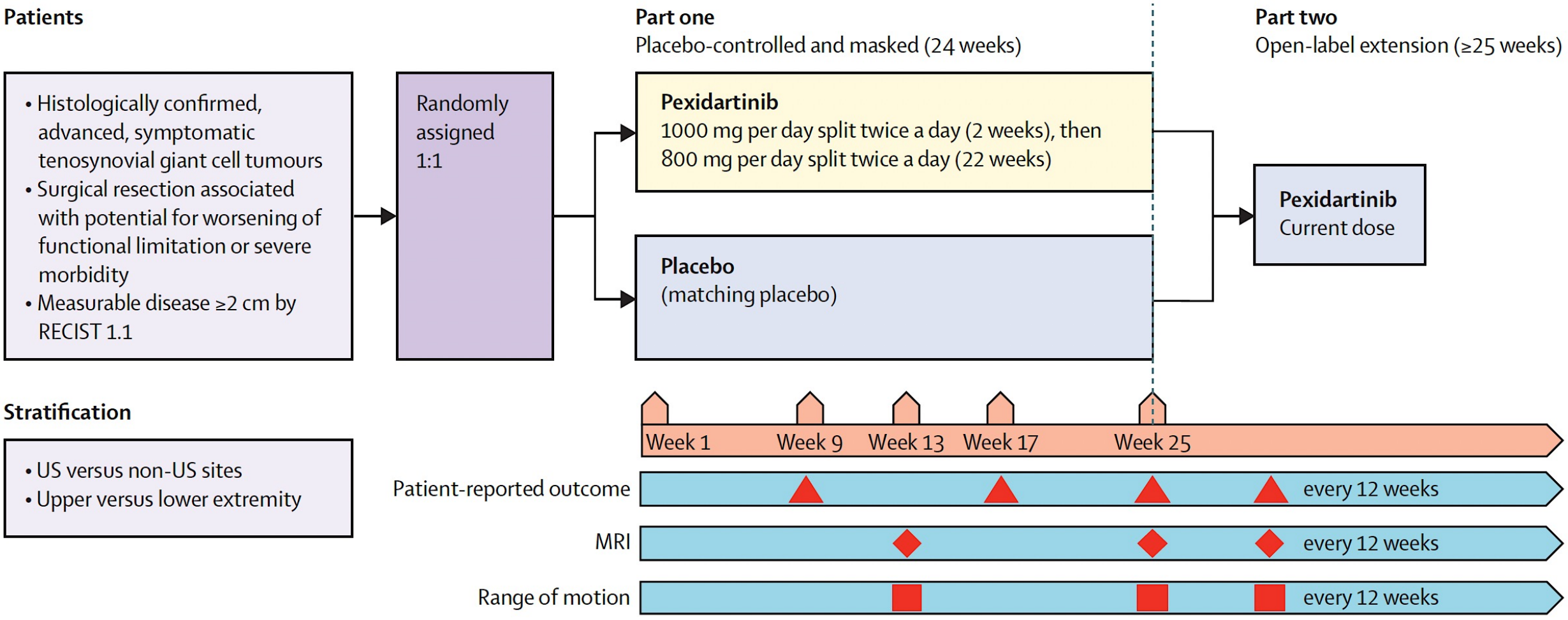
Paracrine loop recruits inflammatory cells

Autocrine loop drives proliferation of neoplastic cells

***CSF1* gene < 77% in localized type and 75% in diffuse type[2]**

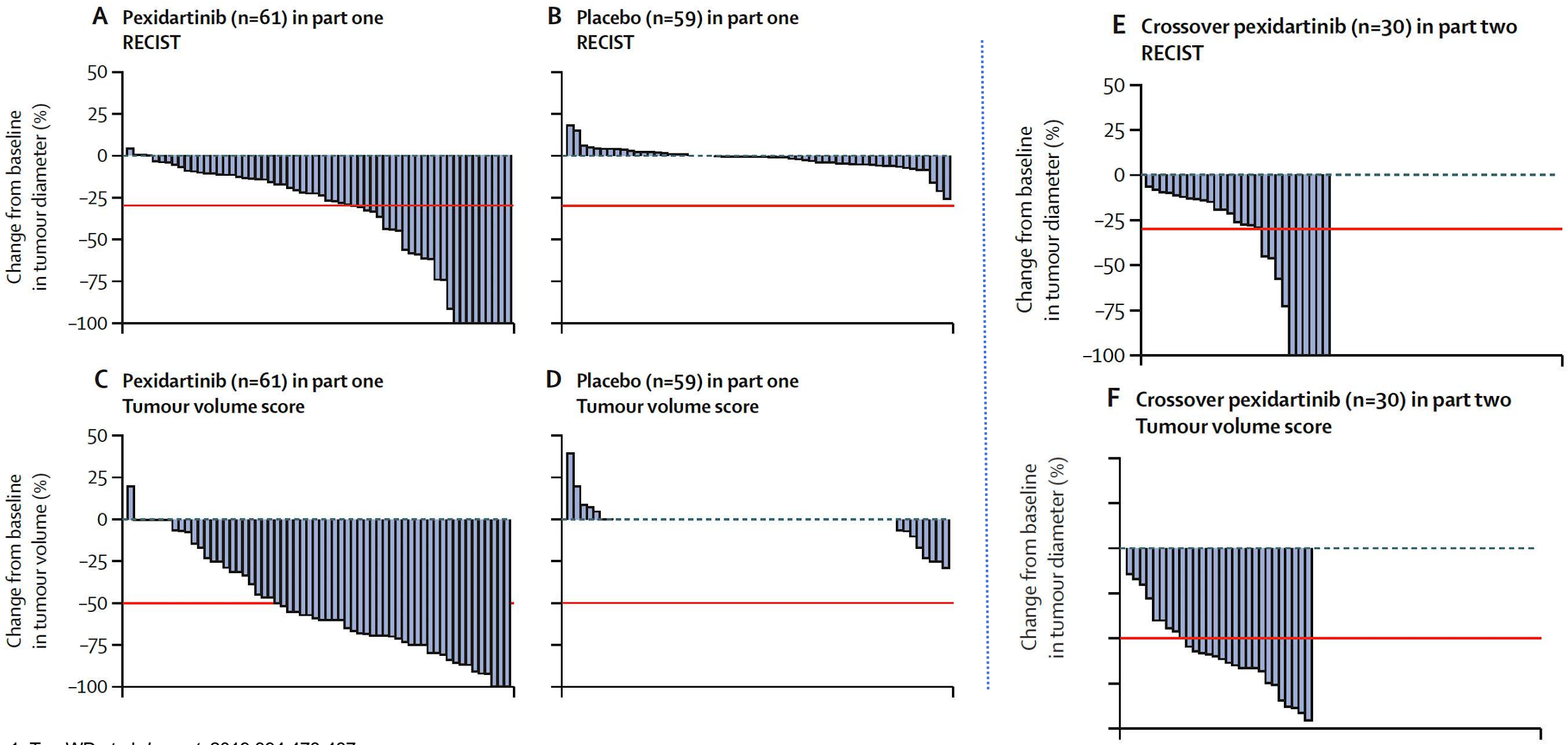
***CSF1* fusions result in the deletion of *CSF1* exon 9, an important negative regulator of CSF-1 expression[3]**

Phase III ENLIVEN Trial: Pexidartinib for Advanced TGCT[1]



1. Tap WD et al. *Lancet.* 2019;394:478-487.

ENLIVEN: Response Based on Tumor Change From Baseline[1]



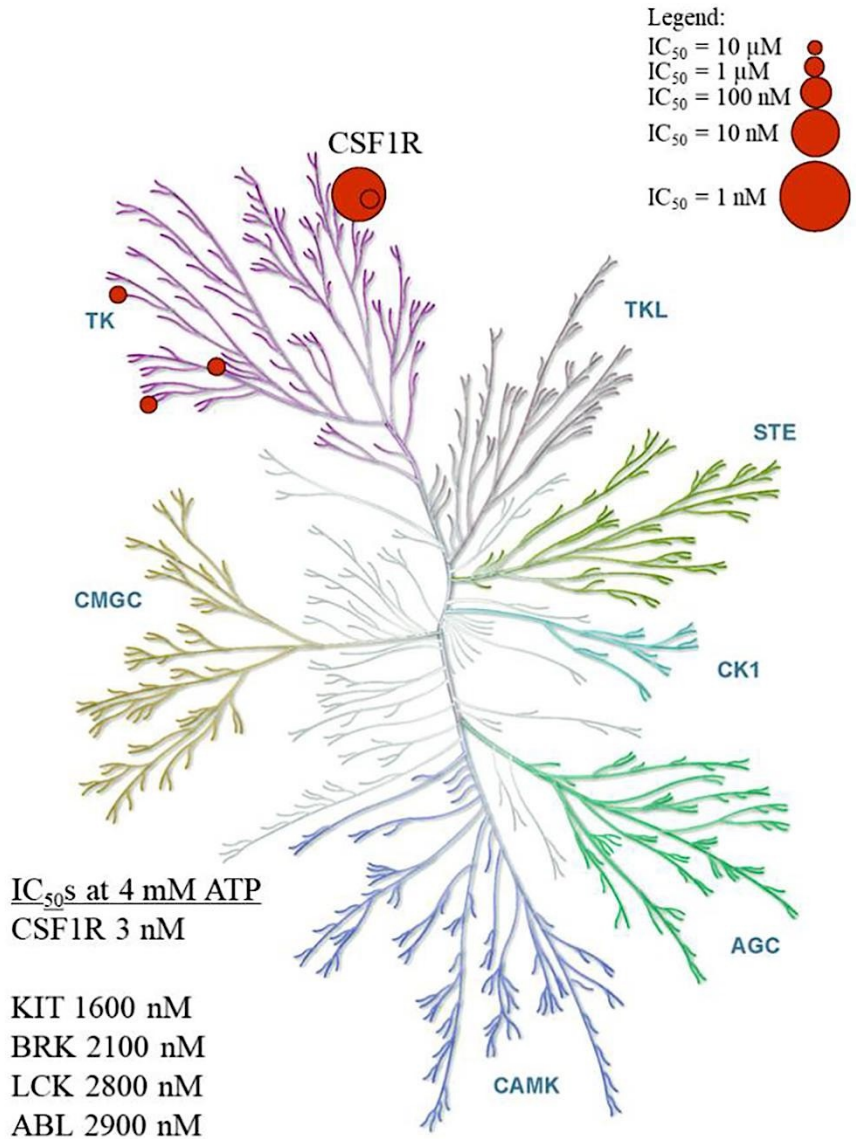
1. Tap WD et al. *Lancet.* 2019;394:478-487.

ENLIVEN: Selected Adverse Events of Interest[1]

	Part one				Part two	
	Pexidartinib (n=61)		Placebo (n=59)		Crossover pexidartinib (n=30)	
	Any grade	Grade 3 or 4	Any grade	Grade 3 or 4	Any grade	Grade 3 or 4
Skin disorders						
Hair colour changes	41 (67%)	0	2 (3%)	0	25 (83%)	0
Gastrointestinal disorders						
Nausea	23 (38%)	0	24 (41%)	0	6 (20%)	0
General disorders						
Fatigue	33 (54%)	0	21 (36%)	0	5 (17%)	0
Investigations						
Aspartate amino-transferase increase	24 (39%)	6 (10%)	0	0	5 (17%)	1 (3%)
Alanine aminotransferase increase	17 (28%)	6 (10%)	1 (2%)	0	7 (23%)	2 (7%)
Alkaline phosphatase increase	9 (15%)	4 (7%)	0	0	1 (3%)	1 (3%)
Lactate dehydrogenase increase	7 (12%)	1 (2%)	0	0	3 (10%)	0

1. Tap WD et al. *Lancet.* 2019;394:478-487.

Vimseltinib is an Oral, Switch-Control TKI That Selectively and Potently Inhibits CSF1R



Caldwell TM et al. Bioorg Med Chem Lett. 2022 Oct 15:74:128928.

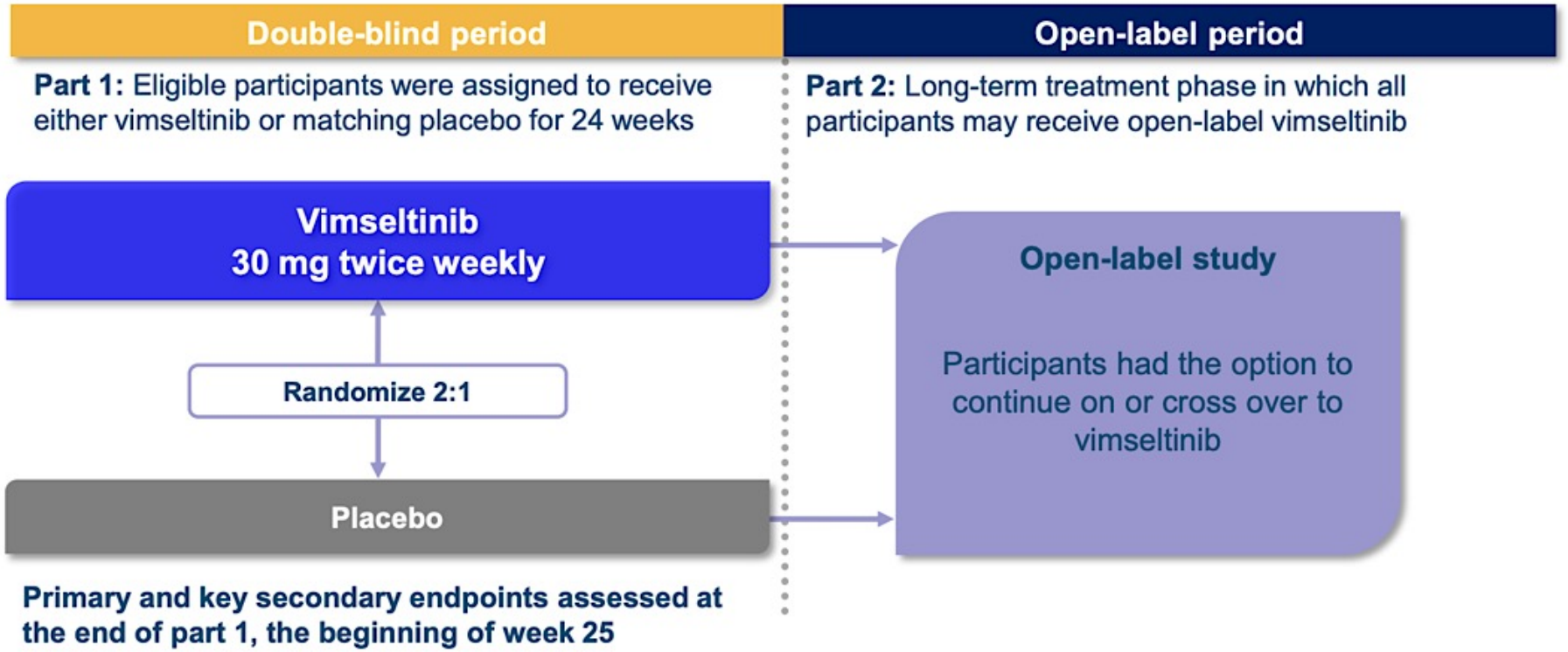
Phase 3 MOTION Study: Safety/Efficacy of Vimseltinib For TGCT Not Amenable to Surgical Resection

Key eligibility criteria

Participants ≥18 years old with a confirmed diagnosis of symptomatic TGCT for which surgical resection would potentially cause worsening functional limitation or severe morbidity

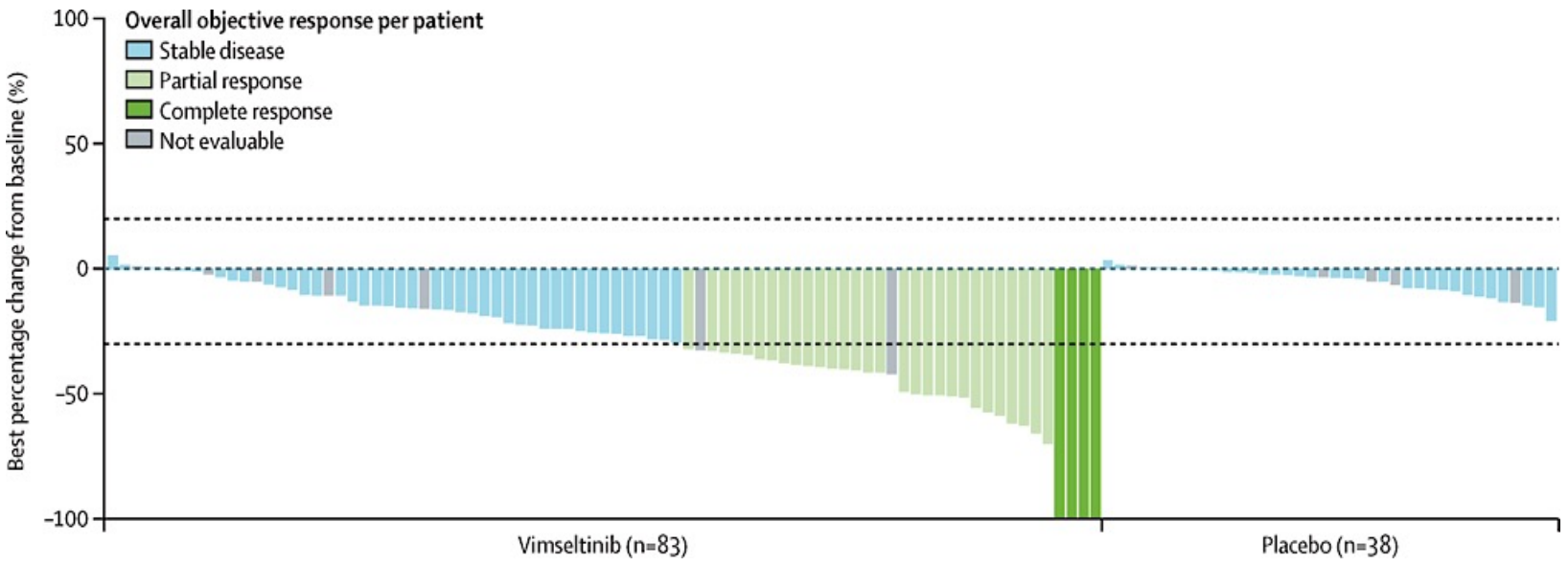
Previous treatment with imatinib or nilotinib was allowed

Randomization was stratified by geographical region and tumor location



Primary endpoint: ORR by independent radiological review (IRR) using RECIST v1.1 at week 25
Powered to detect a 30% difference between treatment arms

Phase 3 MOTION Trial: Vimseltinib Demonstrated Robust and Statistically Significant Antitumor Activity By RECIST v1.1



At week 25	Vimseltinib n = 83	Placebo n = 40
Overall response using RECIST v1.1		
CR	4 (5)	0
PR	29 (35)	0
SD	42 (51)	33 (83)
NE	8 (10)	7 (18)
ORR using RECIST v1.1	33 (40)	0
Treatment difference, % (95% CI), *P*-value[a]	40 (29 to 51), ***P* <0.0001**	
DOR using RECIST v1.1, months, median[b] (min, max)	NR (0.03+,11.7+)	N/A

ORR per RECIST was 40% in the vimseltinib group *vs* 0% in the placebo group (difference 40% [95% CI 29–51]; p<0·0001)

Tap WD et al. Future Oncol. 2024 Mar;20(10):593-601.
Gelderblom H et al. Lancet. 2024 Jun 22;403(10445):2709-2719.

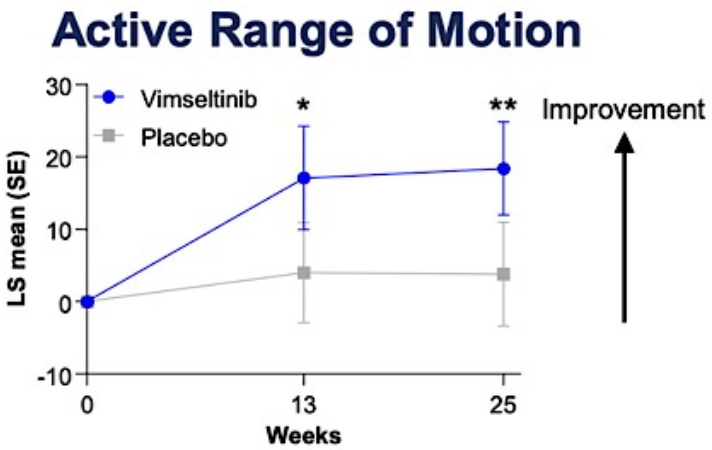
Phase 3 MOTION Trial: Vimseltinib Demonstrated Statistically Significant Antitumor Activity By Tumor Volume Score

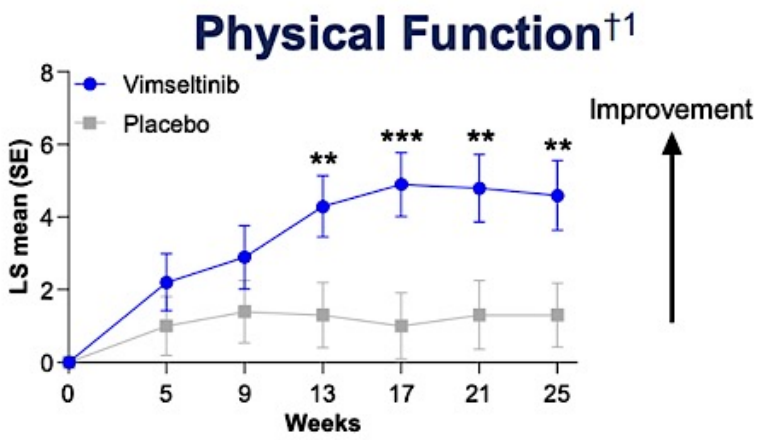
- The irregular growth and shape of TGCT can make measurement with linear methods, like RECIST, difficult
- Tumor Volume Score (TVS) is a TGCT-specific semiquantitative MRI scoring system that estimates tumor volume
- TVS response corresponds to ≥50% reduction in tumor volume
- Response by TVS at week 25 may predict long-term response by RECIST

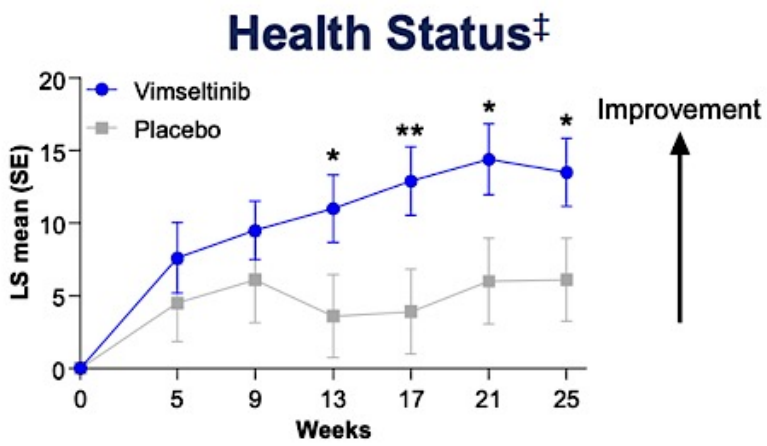
At week 25	Vimseltinib n = 83	Placebo n = 40
Overall response using TVS		
CR	4 (5)	0
PR	52 (63)	0
SD	19 (23)	34 (85)
PD	0	1 (3)
NE	8 (10)	5 (13)
ORR using TVS	56 (67)	0
Treatment difference, % (95% CI), *P*-value	67 (56 to 77) ***P* <0.0001**	
DOR using TVS, months, median[a] (min, max)	NR (0.03+, 13.9+)	N/A

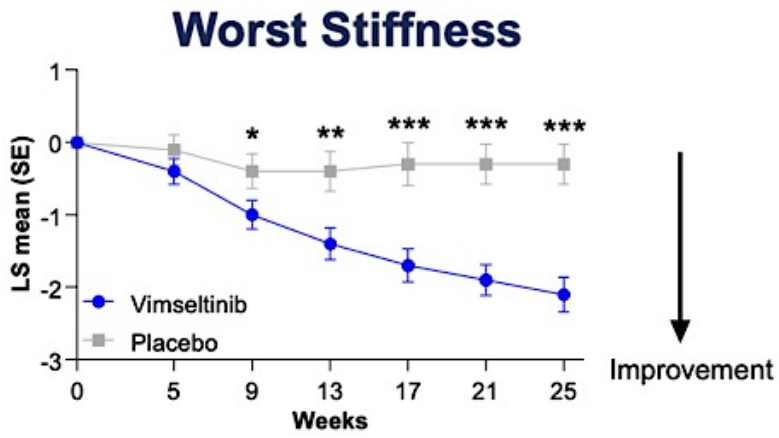
Tap WD et al. Future Oncol. 2024 Mar;20(10):593-601.
Gelderblom H et al. Lancet. 2024 Jun 22;403(10445):2709-2719.

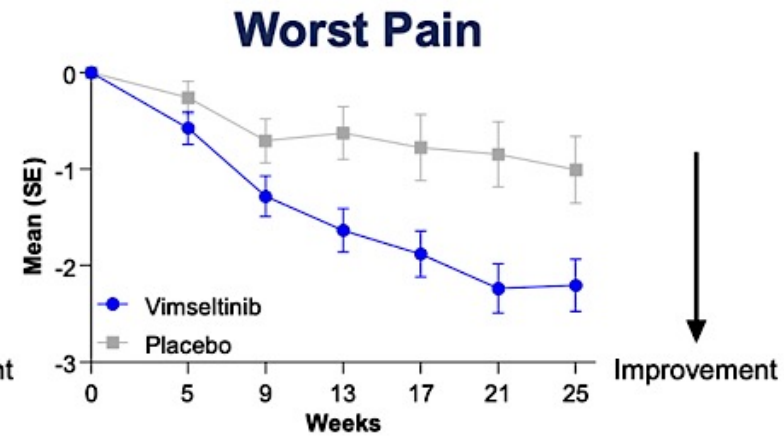
Patient-Reported Outcomes Showed That Vimseltinib Provided Early and Durable Functional and Symptomatic Improvements











Regardless of objective tumor response by IRR using RECIST v1.1, approximately 40% of participants receiving vimseltinib achieved a response in ≥3 clinical outcomes vs 6% of participants receiving placebo

Vimseltinib Was Generally Well Tolerated With Few Discontinuations Due to TEAEs

TEAEs in ≥15% of participants in either treatment arm	Vimseltinib n = 83		Placebo n = 39[a]	
Preferred term, n (%)	All grades	Grade 3/4	All grades	Grade 3/4
Periorbital edema	37 (45)	3 (4)	5 (13)	0
Fatigue	27 (33)	0	6 (15)	0
Face edema	26 (31)	1 (1)	3 (8)	0
Pruritus	24 (29)	2 (2)	3 (8)	0
Headache	23 (28)	1 (1)	10 (26)	0
Asthenia	22 (27)	1 (1)	9 (23)	1 (3)
Nausea	21 (25)	0	8 (21)	1 (3)
Blood CPK increased	20 (24)	8 (10)	0	0
AST increased	19 (23)	0	1 (3)	0
Arthralgia	16 (19)	0	6 (15)	1 (3)
Rash	16 (19)	0	2 (5)	0
Rash maculopapular	16 (19)	1 (1)	0	0
Edema peripheral	15 (18)	0	3 (8)	0
Hypertension	14 (17)	4 (5)	4 (10)	1 (3)
Diarrhea	10 (12)	0	8 (21)	1 (3)

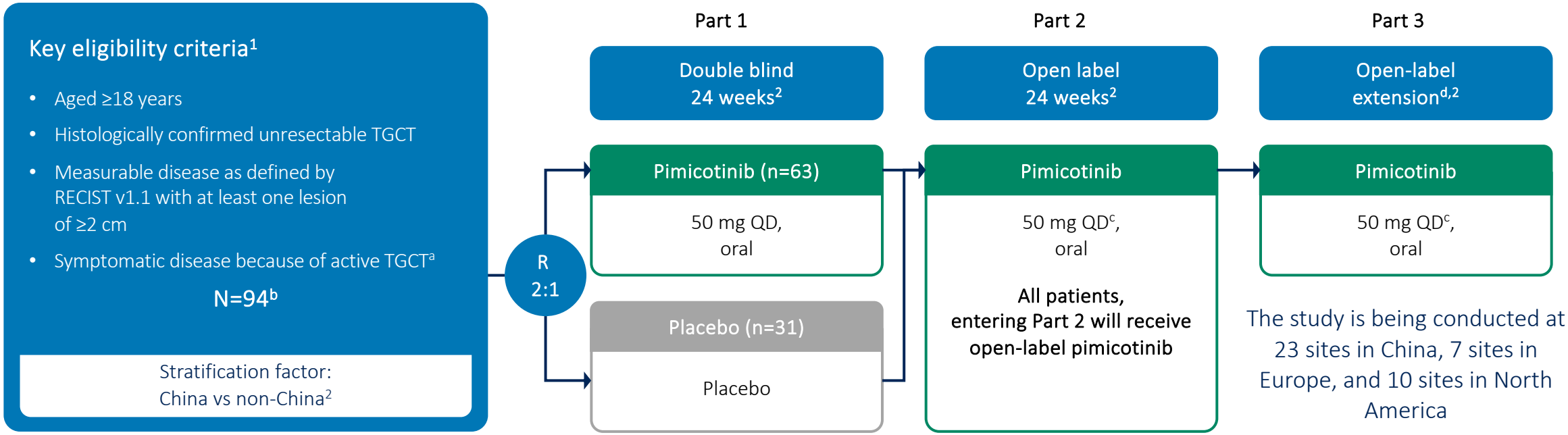
- Most TEAEs were grade 1/2
- Serum enzyme elevations were consistent with the known mechanism of action of CSF1R inhibitors
- TEAEs led to treatment discontinuation in 6% of participants receiving vimseltinib
- There was no evidence of cholestatic hepatotoxicity, drug-induced liver injury, or hair/skin hypopigmentation

Tap WD et al. Future Oncol. 2024 Mar;20(10):593-601.
Gelderblom H et al. Lancet. 2024 Jun 22;403(10445):2709-2719.

Methods

MANEUVER: A Phase 3, randomized, double-blind, placebo-controlled global study of pimicotinib in TGCT

Study design



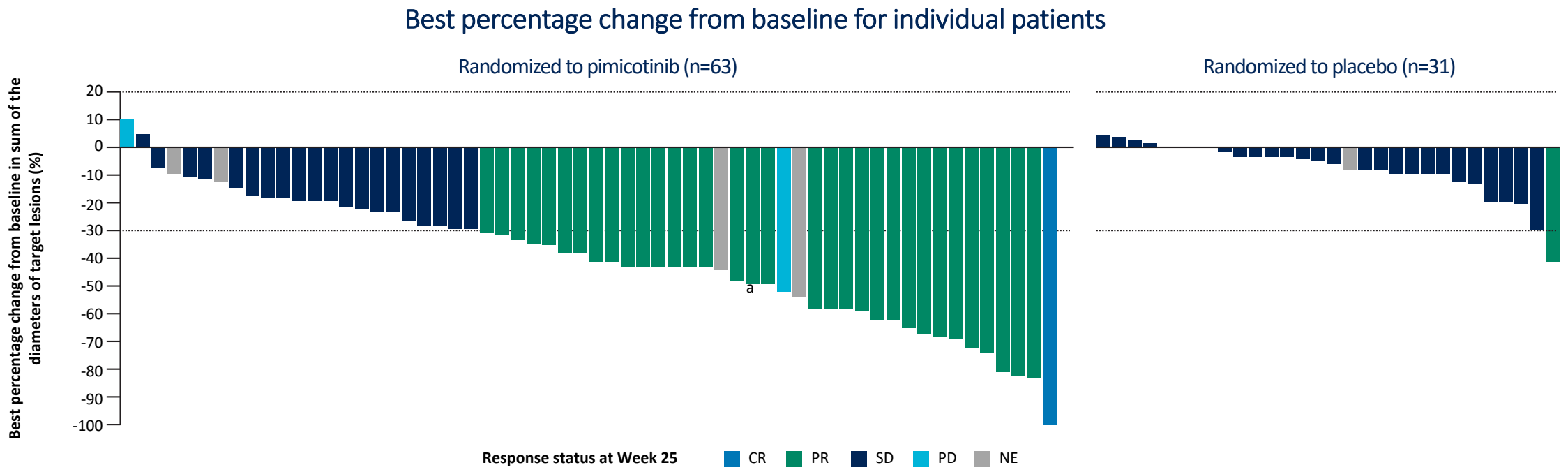
[a]Defined as one or more of the following: (i) a worst pain of ≥4 within 2 weeks prior to randomization (based on scale of 0 to 10, with 10 representing "pain as bad as you can imagine"), (ii) a worst stiffness of ≥4 within 2 weeks prior to randomization (based on a scale of 0 to 10, with 10 representing "stiffness as bad as you can imagine); [b]Between April 27, 2023 and March 29, 2024, 94 adults with TGCT underwent randomization: 63 were assigned to pimicotinib 50 mg QD and 31 to matching placebo; [c]If a patient has dose modification in Part 1/Part 2, the patient will continue to be administered at the modified dose in Part 2/Part 3; [d]All patients who complete 24 weeks of dosing in Part 2 will be eligible to enter the open-label extension treatment phase (ie, Part 3) for a longer treatment period and safety follow-up; R, randomization

1. ClinicalTrials.gov. https://clinicaltrials.gov/study/NCT05804045 [Accessed October 24, 2024]; 2. Niu X et al. Future Oncol 2024;1–8

Results

Pimicotinib resulted in substantial reductions in tumor size

By the data cutoff, 58 of 63 patients (92.1%) in the pimicotinib group had a decrease in tumor size per BIRC based on RECIST v1.1



Data cutoff date Sep 23, 2024

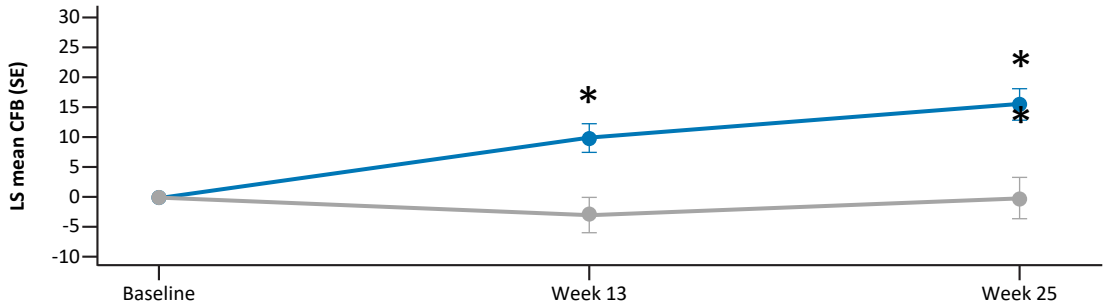
[a]This patient initially experienced a decrease in tumor size of 52% (PR) by Week 13 and then a subsequent increase of 38% (PD) at Week 25; however, by Week 37 the tumor size had reduced by 62% (PR), and patient was still on treatment

Niu X et al. Future Oncol 2024;1–8

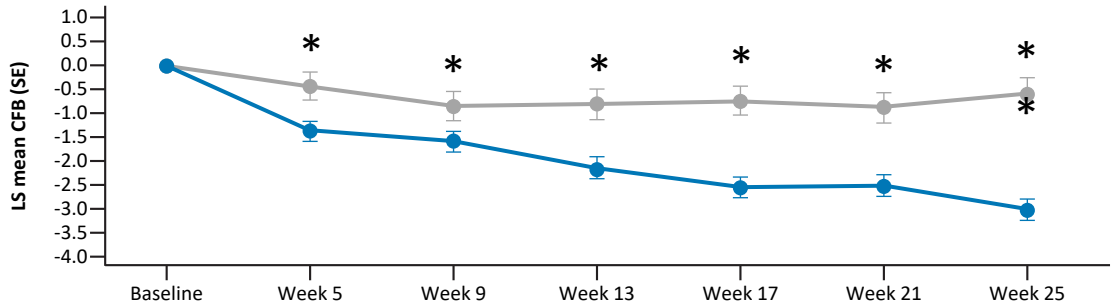
Results

Pimicotinib demonstrated early improvements in relative ROM, worst pain, worst stiffness, and PROMIS-PF T-score

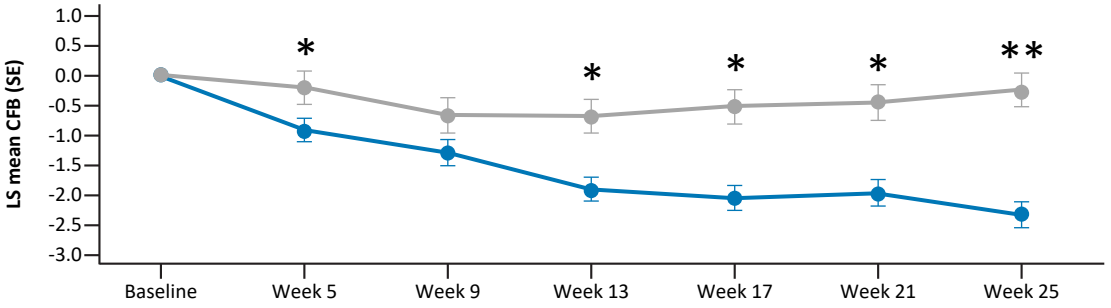
Relative ROM LS mean CFB by visit



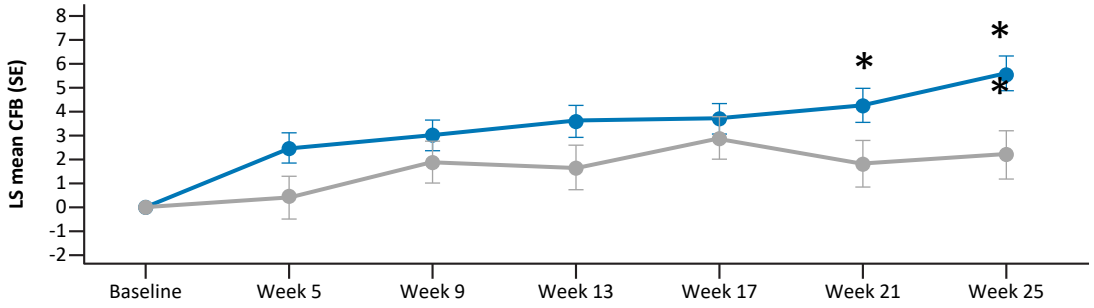
Worst stiffness NRS LS mean CFB by visit



BPI worst pain NRS LS mean CFB by visit



PROMIS-PF T-score LS mean CFB by visit



Treatment: Placebo Pimicotinib

Data cutoff date Sep 23, 2024

*p<0.05 for LS mean group difference at this timepoint; p-values are nominal; **p<0.05 for LS mean group difference at Week 25; p-values are significant (tested in hierarchical order)

CFB, change from baseline; SE, standard error

Results

Most frequent (≥20%) and class-specific TEAEs with pimicotinib

Most common TEAEs (≥20%) by Week 25, n (%)	Pimicotinib n=63		Placebo n=31	
Preferred term	**All grades**	**Grade 3/4**	**All grades**	**Grade 3/4**
Clinical AEs				
Pruritus	33 (52.4)	2 (3.2)	1 (3.2)	0
Face edema	30 (47.6)	0	0	0
Rash	22 (34.9)	2 (3.2)	0	0
Periorbital edema	20 (31.7)	0	3 (9.7)	0
Fatigue	18 (28.6)	0	7 (22.6)	0
Nausea	17 (27.0)	0	2 (6.5)	0
Headache	13 (20.6)	0	2 (6.5)	0

Most common TEAEs (≥20%) by Week 25, n (%)	Pimicotinib n=63		Placebo n=31	
Preferred term	**All grades**	**Grade 3/4**	**All grades**	**Grade 3/4**
Laboratory AEs[a]				
Blood CPK increased	45 (71.4)	8 (12.7)	5 (16.1)	0
Blood LDH increased	36 (57.1)	0	0	0
AST increased	34 (54.0)	0	3 (9.7)	0
Amylase increased	22 (34.9)	0	0	0
α-HBDH increased	16 (25.4)	0	0	0
Lipase increased	15 (23.8)	2 (3.2)	1 (3.2)	0

- There was no evidence of hair/skin hypopigmentation
- TEAEs of hypertension occurred in 14.3% of patients treated with pimicotinib (Grade 3, 3.2%)

- In the pimicotinib arm, AST/ALT elevations were mainly Grade 1 (50.8%/15.9%; Grade 2 3.2%/1.6%), and there was no evidence of cholestatic hepatotoxicity or drug-induced liver injury

Data cutoff date Sep 23, 2024; [a]Laboratory abnormalities were all asymptomatic and responded well to brief dose interruptions. Asymptomatic serum enzyme elevations were consistent with the known mechanism of action of CSF-1R inhibitors;
α-HBDH, alpha hydroxybutyrate dehydrogenase; AE, adverse event; ALT, alanine aminotransferase; AST, aspartate aminotransferase; CPK, creatine phosphokinase; LDH, lactate dehydrogenase

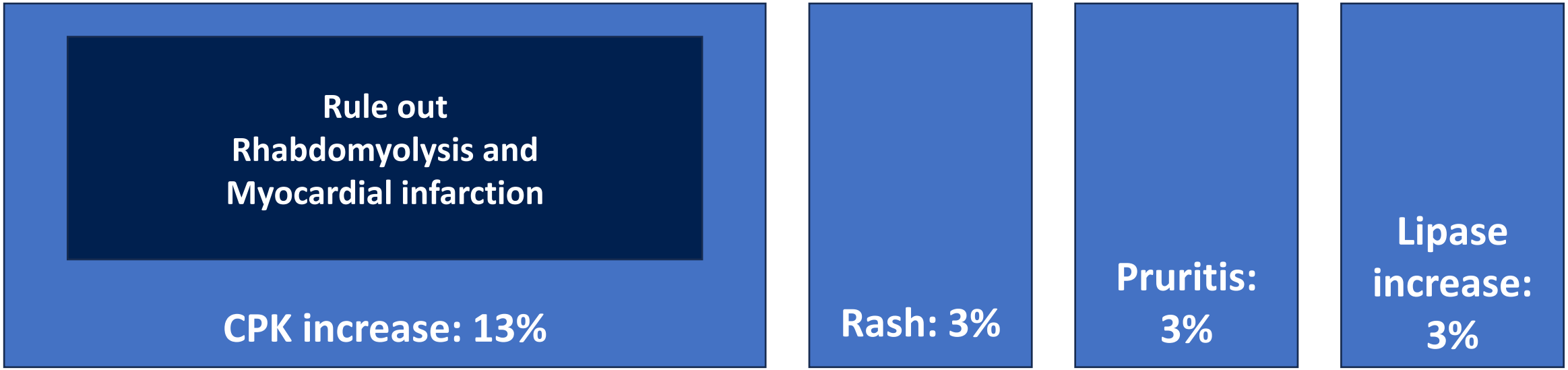
Niu X et al. Future Oncol 2024;1–8

Landscape of published phase III trials

Trials	ENLIVEN (n=100) [1]	MOTION (n=123) [2]	MANEUVER (n=94) [3]
TKI	Pexidartinib	Vimseltinib	Pimicotinib
ORR at wk 24 [95%-CI]			
R of Motion Stiffness Pain			
Grade ≥3 [95%-CI]			
Dose reduction [95%-CI]			
Discontinuation [95%-CI]			

(1) – Tap et al. Lancet. 2019; (2) – Gelderblom et al. Lancet Oncol 2024; (3) Niu et al. ASCO 2025

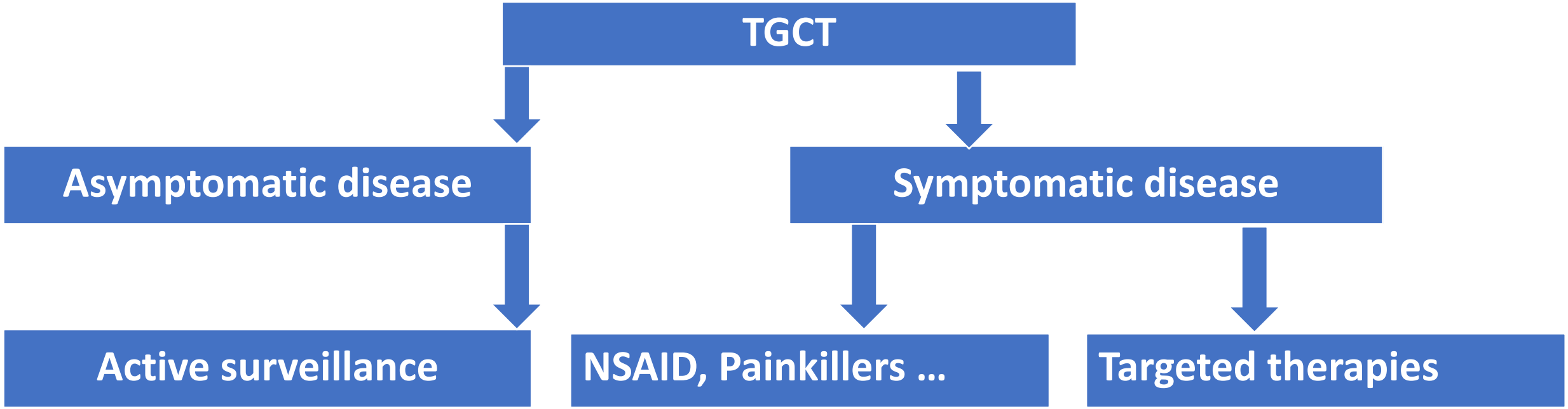
Grade≥3 toxicities in the context of benign tumour



Trials	ENLIVEN (n=100) [1]	MOTION (n=123) [2]	MANEUVER (n=94) [3]
TKI	Pexidartinib	Vimseltinib	Pimicotinib
Grade≥3 CPK increase	**Not reported**	**10%**	**13%**

(1) – Tap et al. Lancet. 2019; (2) – Gelderblom et al. Lancet Oncol 2024; (3) Niu et al. ASCO 2025

Management of tenosynovial giant cell tumor



Vimseltinib (FDA: Yes/EMA: Yes)
Pimicotinib … ASAP ?
Pexidartinib (FDA: Yes/EMA: No)

Adapted from Stacchiotti S et al. Cancer Treat Rev. 2023

Agenda

Introduction: Current Role of General Medical Oncologists in the Treatment of Soft Tissue Sarcomas (STS)

Module 1: Incorporation of Novel Agents and Strategies into the Management of STS — Faculty Presentation

Module 2: Incorporation of Novel Agents and Strategies into the Management of STS — Survey Questions

Module 3: Evolving Treatment Paradigm for Locally Aggressive STS — Faculty Presentation

Module 4: Evolving Treatment Paradigm for Locally Aggressive STS — Survey Questions

Module 5: ASCO 2025

Questions from General Medical Oncologists — Desmoid Tumors: Observation vs treatment

- Often patients are initially observed. When do you decide to initiate therapy?
- When to observe desmoid tumors?
- Would you ever consider surgery in desmoid tumors upfront?
- How effective is radiation therapy for desmoid tumors as a therapeutic modality? What about as palliation?
- Do you send all your patients with desmoid tumors for genetic counseling?

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Questions from General Medical Oncologists — Desmoid Tumors: Systemic Treatment

- How effective is liposomal doxorubicin in the treatment of desmoid tumors?
- How does approval of nirogacestat change approach to upfront surgery vs drug therapy?
- Is nirogacestat being studied in a perioperative setting for patients with desmoid tumor, especially those with intraabdominal sites that are not easily amenable for surgery?

Questions from General Medical Oncologists — Desmoid Tumors: Nirogacestat

- 19-year-old woman with symptomatic pelvic desmoid tumor x 5 years relatively stable on low dose sorafenib on and off but now larger with more pain. What do you recommend next? If you decide to give nirogacestat, what do you advise her regarding ovarian function? What about upfront egg harvesting?
- How to manage use in premenopausal patients about to start nirogacestat regarding ovarian dysfunction?

Questions from General Medical Oncologists — Desmoid Tumors: Nirogacestat

- What patient or tumor characteristics make someone a good candidate for nirogacestat, and how does this therapy compare with prior approaches in terms of tolerability and impact on symptom burden?
- How do you manage diarrhea with nirogacestat? Dose reduction, loperamide, octreotide?
- How best to manage AEs? I had a patient stop treatment due to AEs.

Questions from General Medical Oncologists — TGCT: CSF1R Inhibitors

- What are the key similarities and differences among available and experimental CSF1R inhibitors for treating TGCT, and how do these factors influence your choice of agent and management strategy?
- How do you choose between vimseltinib and pexidartinib?
- Have you used either agent in the neoadjuvant setting? What about the adjuvant setting?

Questions from General Medical Oncologists — TGCT: CSF1R Inhibitors

- Any difference in vimseltinib and pexidartinib in terms of response and outcome between the nodular and the diffuse types of TGCT?
- Any other TKIs that are effective treating TGCT?
- How often do you manage liver function with pexidartinib?

Questions from General Medical Oncologists — Afamitresgene autoleucel

- Are the toxicities secondary to T-cell receptor gene therapy afamitresgene autoleucel for patients similar to CAR T cell therapy? What unique post-therapy AEs should physicians be mindful of in the community setting among those treated with this novel therapy?
- In patients with advanced synovial sarcoma w/ MAGE-A4 expression, who receive afamitresgene autoleucel therapy at an academic cancer center, how do you manage long-term complications in a small community practice when the patient returns?
- The T-cell receptor gene therapy is a new modality of treatment, so would appreciate information on diagnosis, staging, and treatment algorithms.
- What tox profile and can it be done in community or academic center?

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Questions from General Medical Oncologists — Advanced STS: Sequencing of agents

- Second-line treatment of a malignant peripheral nerve sheath tumor after progression on doxorubicin ifosfamide/ifosfamide etoposide for 8 courses of treatment total (ECOG 0, age 45)?
- Besides chemotherapy what other options are available? I have a patient with recurrent sarcoma that is going to be treated with a thermal IR procedure in his lungs that is thought to develop more TLS formation - is there a role for PD1/CTLA4 inhibition?

Agenda

Introduction: Current Role of General Medical Oncologists in the Treatment of Soft Tissue Sarcomas (STS)

Module 1: Incorporation of Novel Agents and Strategies into the Management of STS — Faculty Presentation

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Module 4: Evolving Treatment Paradigm for Locally Aggressive STS — Survey Questions

Module 5: ASCO 2025

RTP RESEARCH TO PRACTICE

Pimicotinib in tenosynovial giant cell tumor (TGCT): Efficacy, safety and patient-reported outcomes of phase 3 MANEUVER study.

Niu X et al

ASCO 2025; Abstract 11500

Anlotinib in combination with epirubicin followed by maintenance anlotinib versus placebo plus epirubicin as first-line treatment for advanced soft tissue sarcoma (STS): A randomized, double-blind, parallel-controlled, phase III study.

Zhou Y et al.

ASCO 2025; Abstract 11501

Eribulin plus anlotinib in advanced soft tissue sarcoma (ERAS): Updates on efficacy and biomarkers.

Deng Y et al

ASCO 2025; Abstract 11502

ASCO 2025 | ORAL ABSTRACT SESSION

RTP RESEARCH TO PRACTICE

Off-label use of fam-trastuzumab deruxtecan in desmoplastic small round cell tumor.
Slotkin ES et al.
ASCO 2025; Abstract 11504

A randomized phase III trial of catequentinib hydrochloride (AL3818) versus placebo in subjects with metastatic or advanced leiomyosarcoma (LMS).
Jones RL et al.
ASCO 2025; Abstract 11506

Alliance A092104: A randomized phase 2/3 study of olaparib plus temozolomide versus investigator's choice for the treatment of patients with advanced uterine leiomyosarcoma after progression on prior chemotherapy.
Van Tine BA et al.
ASCO 2025; Abstract 11507.

Detecting ctDNA using personalized structural variants to forecast recurrence in localized soft tissue sarcoma (STS).

Park CL et al.

ASCO 2025; Abstract 11511

Phase II of sunitinib plus nivolumab in extraskeletal myxoid chondrosarcoma: Results from the GEIS, ISG, and UCL IMMUNOSARC II Study.

Hindi N et al.

ASCO 2025; Abstract 11513

ImmunoSarc2 (Cohort 7a): A Spanish Sarcoma Group (GEIS) phase Ib trial of epirubicin and ifosfamide plus nivolumab in first line of advanced undifferentiated pleomorphic sarcoma (UPS).

Broto JM et al

ASCO 2025; Abstract 11514 .

A phase 2 study using metronomic gemcitabine, doxorubicin, and docetaxel plus nivolumab in advanced leiomyosarcoma and liposarcoma (NCT04535713).

Ballon J et al.

ASCO 2025; Abstract 11515

Subgroup analysis of the phase 2 part of the RINGSIDE phase 2/3 trial of varegacestat for treatment of desmoid tumors.

Chugh R et al.

ASCO 2025; Abstract 11516

Long-term clinical outcome assessments in patients with tenosynovial giant cell tumor treated with vimseltinib: 1-year results from the MOTION phase 3 trial
Bhadri VA et al.
ASCO 2025; Abstract 11558

Change in T2-weighted signal intensity, change in tumor volume, and exposure-response analysis in the RINGSIDE phase 2 study of varegacestat in patients with desmoid tumors
Gounder M et al.
ASCO 2025; Abstract 11557

A phase 3, randomized, double-blind, placebo-controlled study to assess the efficacy and safety of emactuzumab in patients with tenosynovial giant cell tumor (TANGENT).
Gelderblom H et al.
ASCO 2025; Abstract TPS11584

Thank you for joining us!

Please take a moment to complete the survey currently up on Zoom. Your feedback is very important to us. The survey will remain open for 5 minutes after the meeting ends.

Information on how to obtain CME credit is provided in the Zoom chat room. Attendees will also receive an email in 1 to 3 business days with these instructions.

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