Company update – April

Jakob Lindberg, CEO

April 27, 2020

oncopeptides

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Oncopeptides at a glance

Develops targeted cancer treatments

- Proprietary peptide-conjugated compounds
- Lead compound Melflufen a first-in-class anti-cancer peptide-conjugated drug targeting Multiple Myeloma

Initial focus on Multiple Myeloma

- Significant market opportunity in orphan indication
- Melflufen Phase 2 study, O-12-M1, showed the best MM survival data to date

Application process initiated for accelerated approval in the US

- Target to submit in Q2-20 based on phase 2 study HORIZON
- Triple-class refractory MM

Phase 3 expected to be fully enrolled very soon

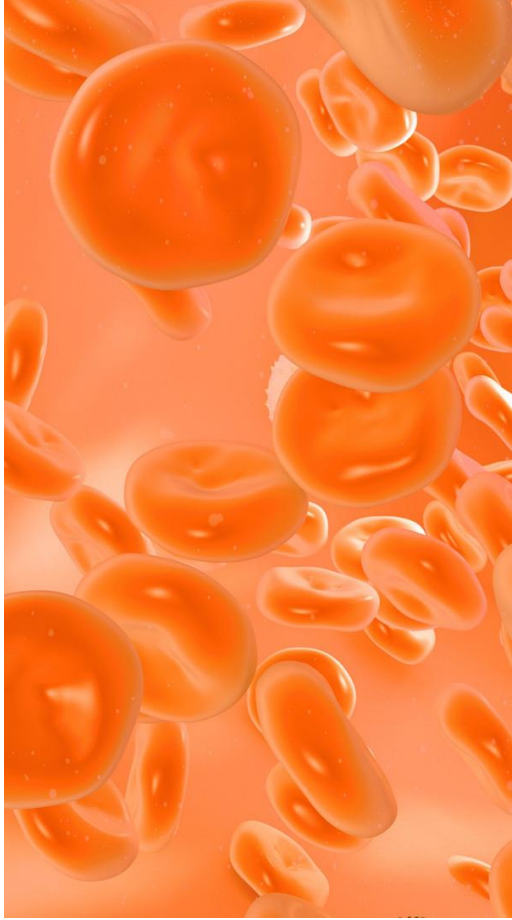
- 423 out of 450 patients recruited as of March 20 at 140 sites
- Two additional supporting studies ongoing. Additional phase 3 called LIGHTHOUSE will start 2020, initiation postponed due to COVID-19

New indications and NCEs in development

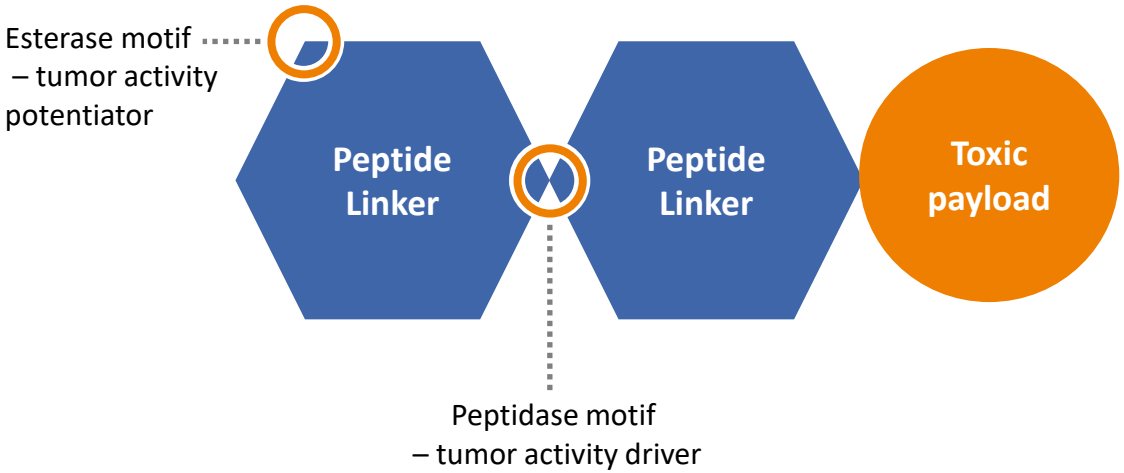
- A Phase 1/2 study addressing AL amyloidosis initiated but paused

Listed on NASDAQ Stockholm, strong financial position

- Market cap: SEK ~7 B
- Cash position: SEK 926 M as of December 31

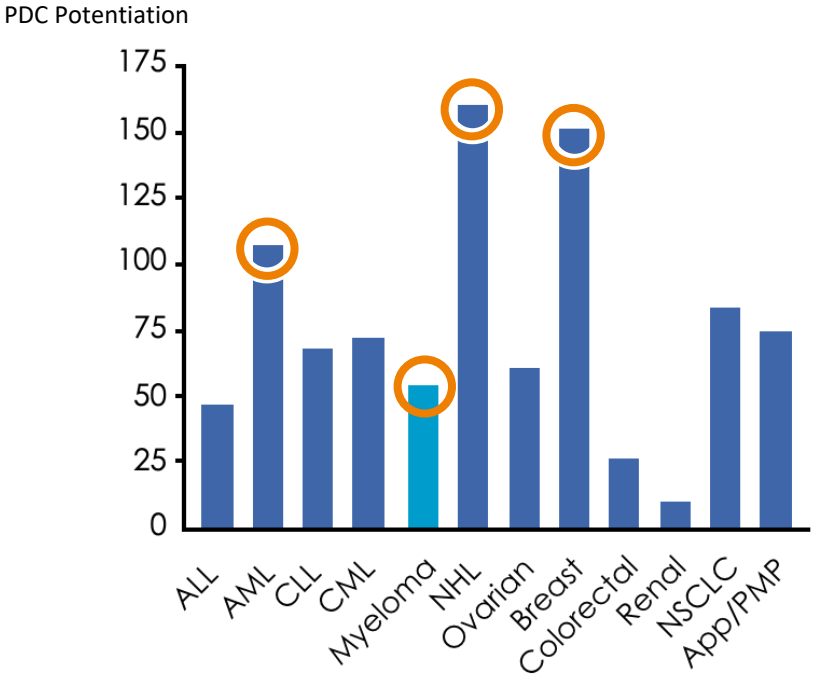


Unique Peptide-Drug Conjugate (PDC) platform



- Targeted delivery of toxins to tumor cells
- Utilizing unique enzymatic motifs
  - Peptidase motif necessary
  - Esterase motif potentiating

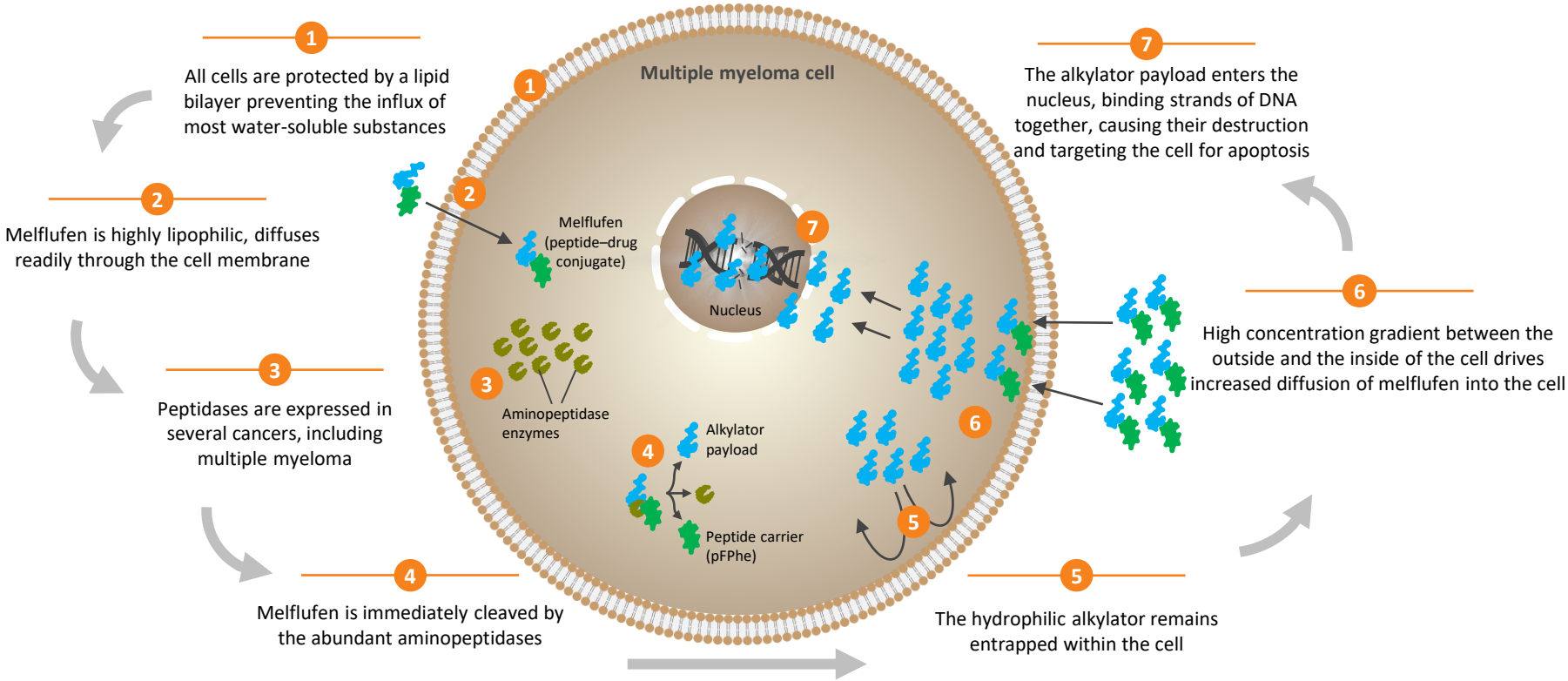
PDC platform exhibits significant therapeutic activity across several cancers



- The PDC platform show good activity across a majority of cancers (data to the left on patient material)
- Based on the PDC platform, Oncopeptides have developed a portfolio of novel molecules
- Lead compound melflufen is focused on multiple myeloma and AL amyloidosis
- Indication expansion focus will be patients suffering from AML, NHL and breast cancer

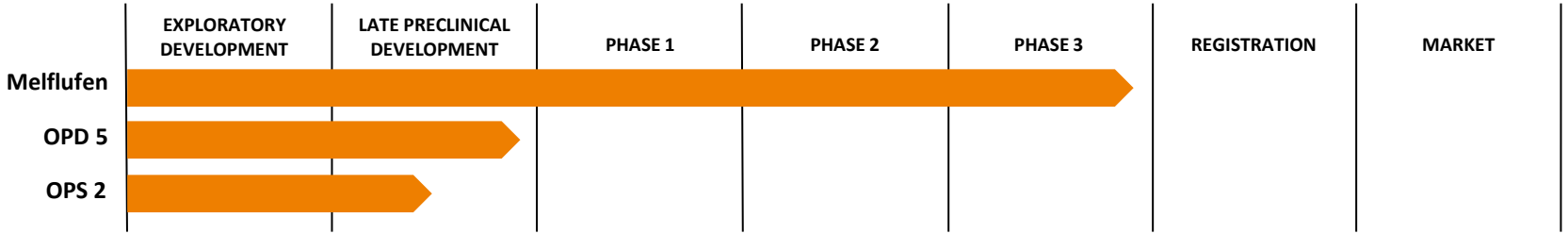
Melflufen is a first-in-class peptide-drug conjugate

- Uses high peptidase levels to target myeloma cells



Chauhan D, et al. Clin Cancer Res 2013;19(11):3019-3031; Wickström M, et al. Invest New Drugs 2008;26(3):195-204; Ray A, et al. Br J Haematol 2016;174(3):397-409; 4. Strese S, et al. Biochem Pharmacol 2013;86(7):888-895; Wickström M, et al. Oncotarget 2017; 8(39):66641-66655.

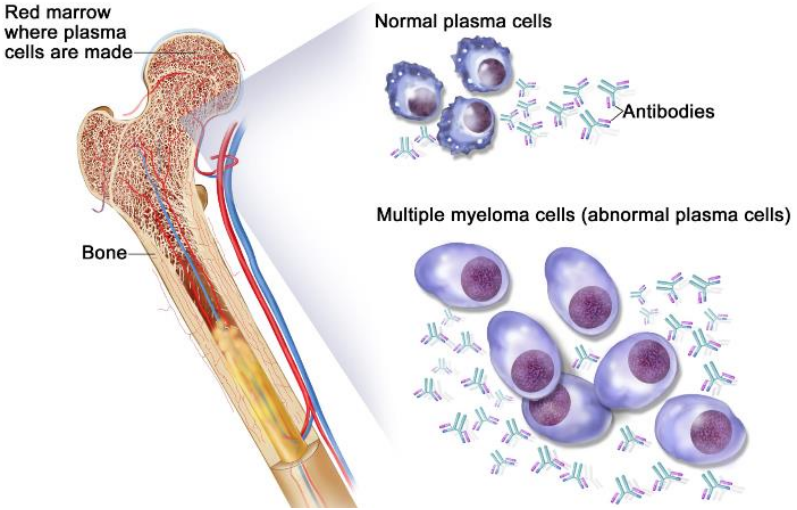
Melflufen is currently in phase 3 - two more PDC candidates ready for the clinic in 2020 and 2021



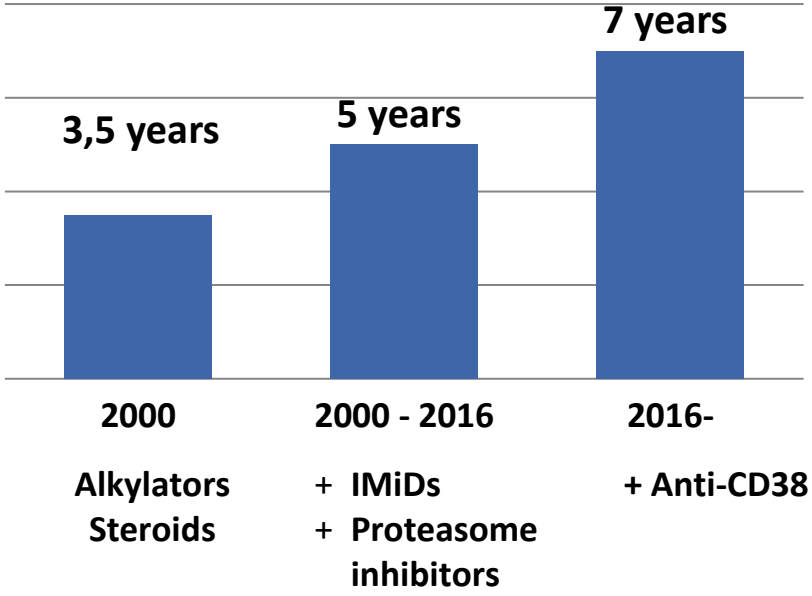
- OPD5 and OPS2 will be ready for the clinic in 2020 and 2021 respectively
  - OPD5 – specialized alkylating PDC candidate for high-dose treatment of patients (i.e. bone-marrow transplantation)
  - OPS2 – second generation PDC compound with an alkylating payload
- Both are novel molecules with composition of matter patents
- Full optionality to fully explore the PDC platform in 2021 and beyond

Multiple Myeloma is a hematological cancer without cure

Myeloma – Uncontrolled plasma cell proliferation



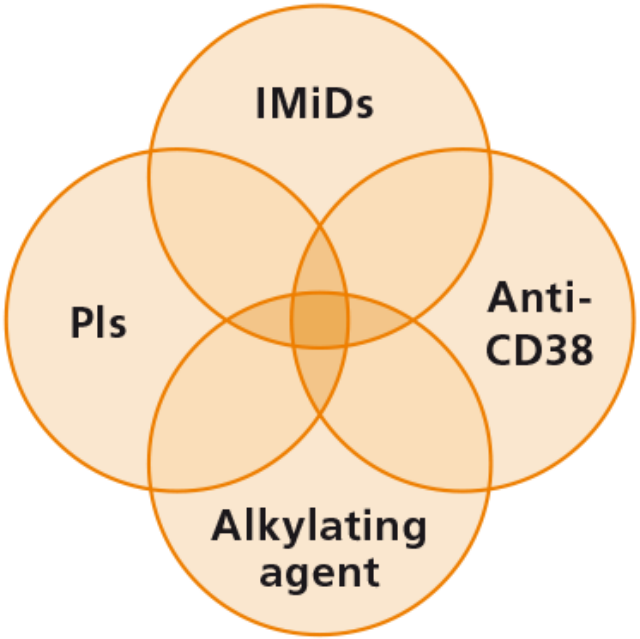
Median Survival increasing with more available treatment options



Source: IntrinsiQ and Kantar Health.

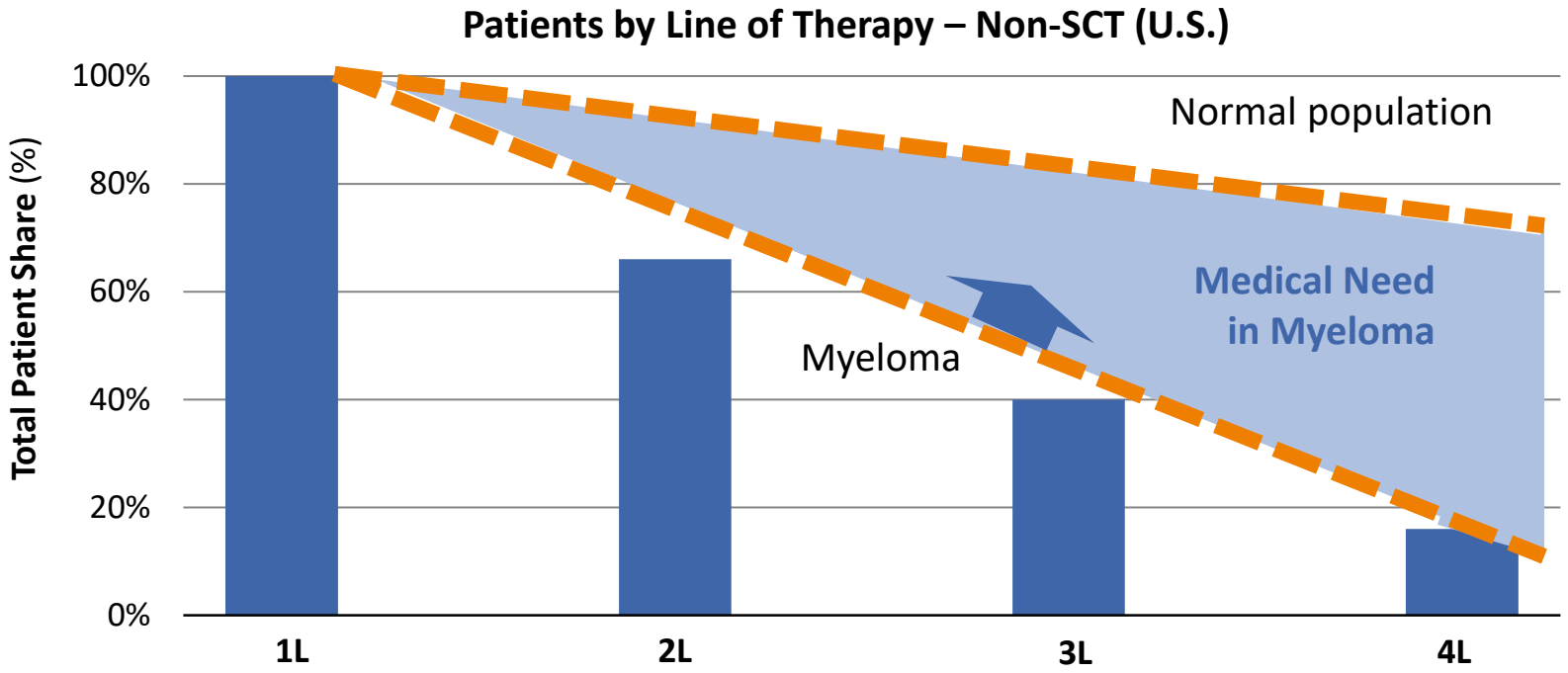
Significant medical needs remain

- Four treatment modalities used with inevitable resistance development
- Currently, the majority of patients have been treated with all four modalities after 2-3 lines of therapy with limited treatment options left
- Frequent co-morbidities further compounding the problem with limited treatment options



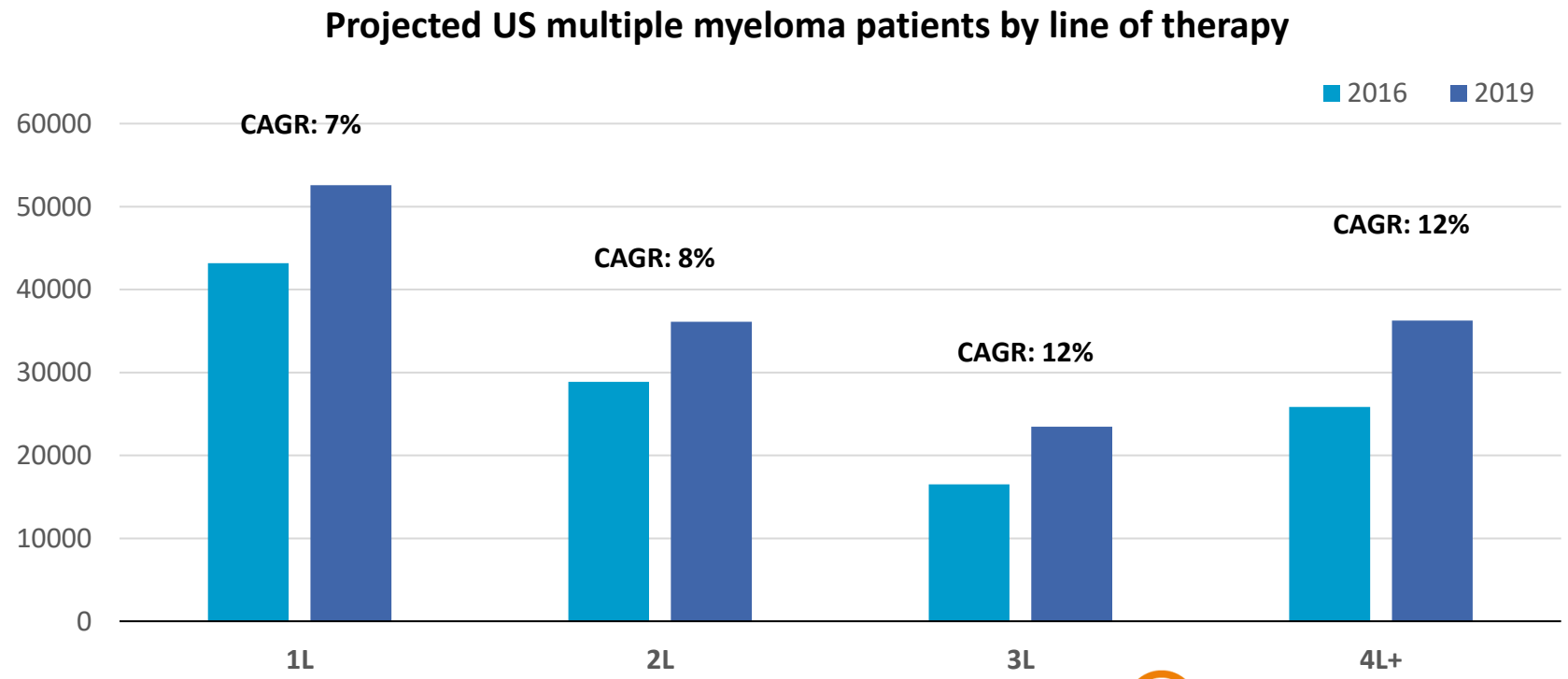
We are still far from making myeloma a chronic disease

– Later line patient population growing with significant need for new treatments



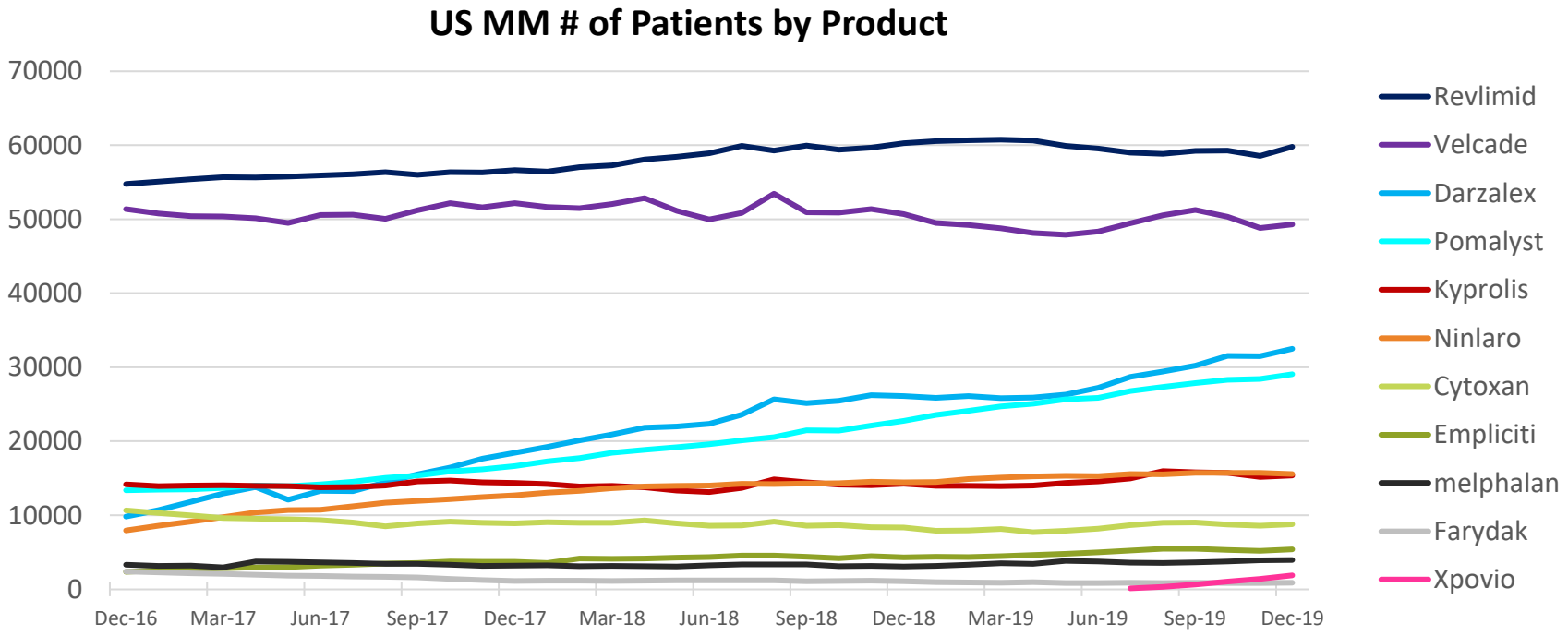
Source: Kantar Health 2018

Improved outcomes lead to fast growth in number of treated patients in later lines of therapy



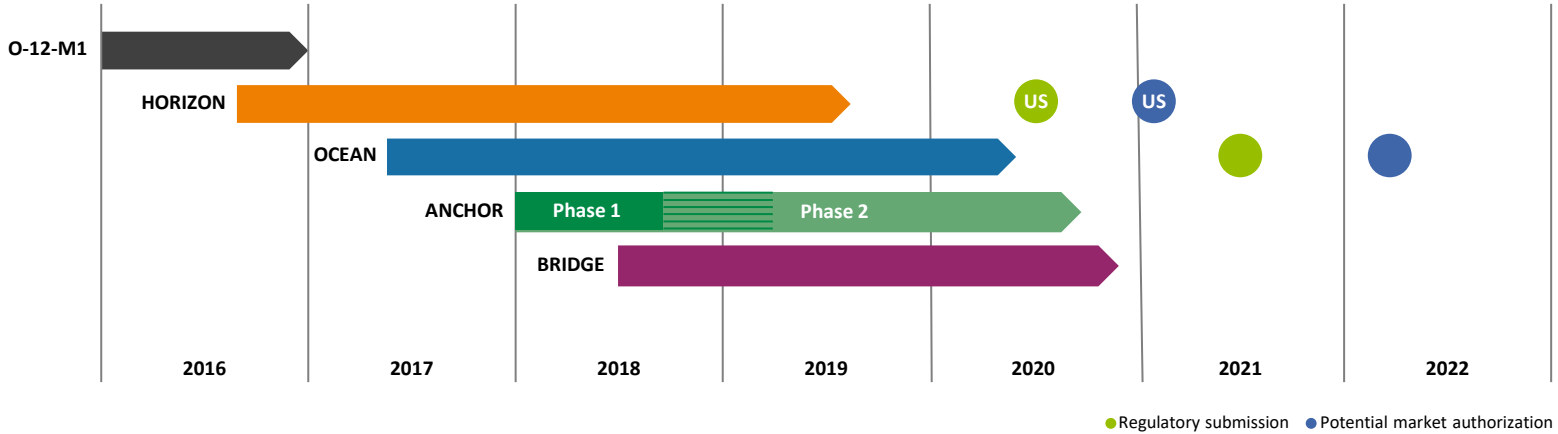
Source: Intrinsiq MAT 2019
Note: 3-yr annual growth rate for 4Q2016-4Q2019

Newer products used in addition to older products as survival improves



Source: Intrinsiq MAT, Dec 2019

Overview of our present clinical development program in multiple myeloma



O-12-M1
Show single-agent activity in RRMM

HORIZON
Show single-agent activity in RRMM

OCEAN
Show single-agent superiority over SoC backbone in RRMM (pomalidomide)

ANCHOR
Show combination synergy and tolerability with daratumumab and bortezomib

BRIDGE
Show that melflufen can beused in patients with renal impairment

Requirements for success in Relapsed Refractory Multiple Myeloma

MUST HAVE CHARACTERISTICS

- Single agent +/- steroid activity in multi-refractory patients of >20% Overall Response Rate
- Single agent +/- steroid approval in refractory patients
- Efficacy synergy in combination with other main myeloma drugs with good tolerability
- No major quality of life/ tolerability issues
- No co-morbidity limitations

NICE TO HAVE CHARACTERISTICS

- Easy administration schedule

Proven single agent activity

Pomalyst

DARZALEX

Comorbidity or tolerability limitations

Kyprolis

FARYDAK (panobinostat) capsules 10mg/15mg/20mg

Limited to no single agent data

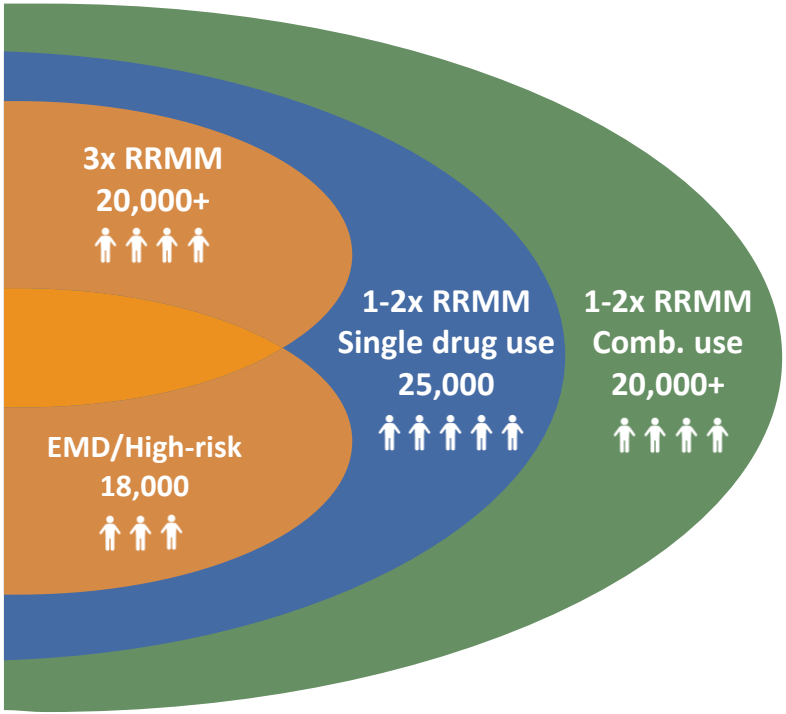
NINLARO

Empliciti (elotuzumab)

Melflufen development program designed to support potential as a new agent after IMiD and PI failure

MUST HAVE CHARACTERISTICS	MELFLUFEN
Single agent +/- steroid activity in multi-refractory patients of >20% Overall Response Rate	O-12-M1 showed an ORR of 31% and HORIZON an ORR of 30% in multi-refractory patients
Single agent +/- steroid approval in refractory patients	OCEAN head to head study vs. Pomalyst/dex is designed for approval
Efficacy synergy in combination with other main myeloma drugs with good tolerability	ANCHOR shows excellent synergy and good tolerability with daratumumab and bortezomib (early data)
No major quality of life/ tolerability issues	Good QoL with almost no non-hematological AEs
No co-morbidity limitations	No co-morbidity or drug-drug interactions limitations
NICE TO HAVE CHARACTERISTICS	
Easy administration schedule	One 30-minute infusion every 28 days

The market opportunity is significant for melflufen's planned label journey in RRMM (US patient numbers)



Clinical Program

HORIZON	Anticipated label in triple class refractory patients.
OCEAN	Head to head superiority study with the most commonly used regimen in RRMM. Majority of RRMM patients use single agent +/- steroid.
ANCHOR LIGHTHOUSE	Combination with PI or anti-CD38 opens up 2L+ combination treatment opportunity.

Source: Patient numbers based on IntrinsiQ analysis.

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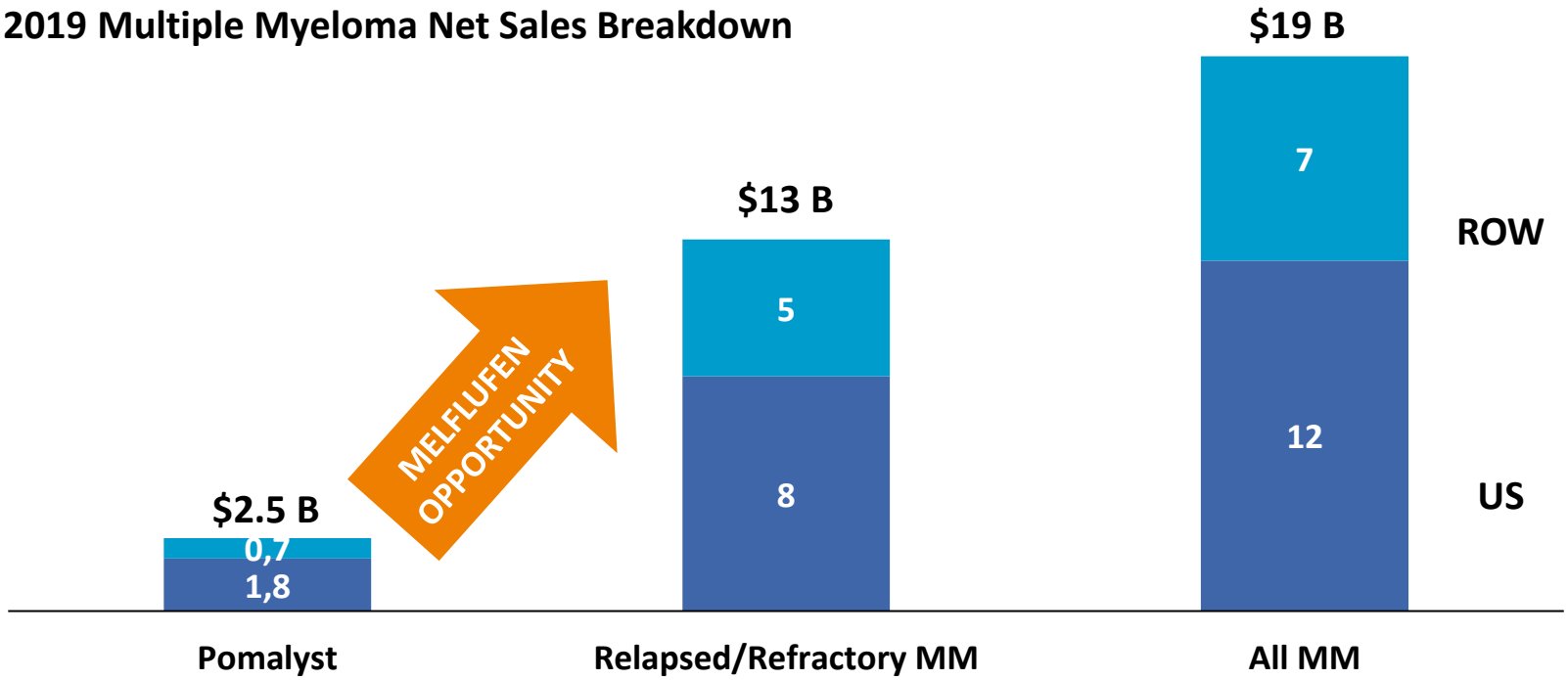
Melflufen opportunity in Relapsed Refractory Multiple Myeloma

– 2019 Multiple Myeloma Net Sales Breakdown



Source: EvaluatePharma, Intrinsiq, company analysis

Recent highlights

COVID-19: Pivotal studies not affected but signal seeking trials affected

- HORIZON not affected
- OCEAN – 423 of 450 patients enrolled, only slight delay in topline results to be expected
- Temporary recruitment pause for bortezomib arm in ANCHOR and in BRIDGE as well as AL Amyloidosis study
- Initiation of new studies such as LIGHTHOUSE postponed

O12-M-1 data published in Lancet Haematology

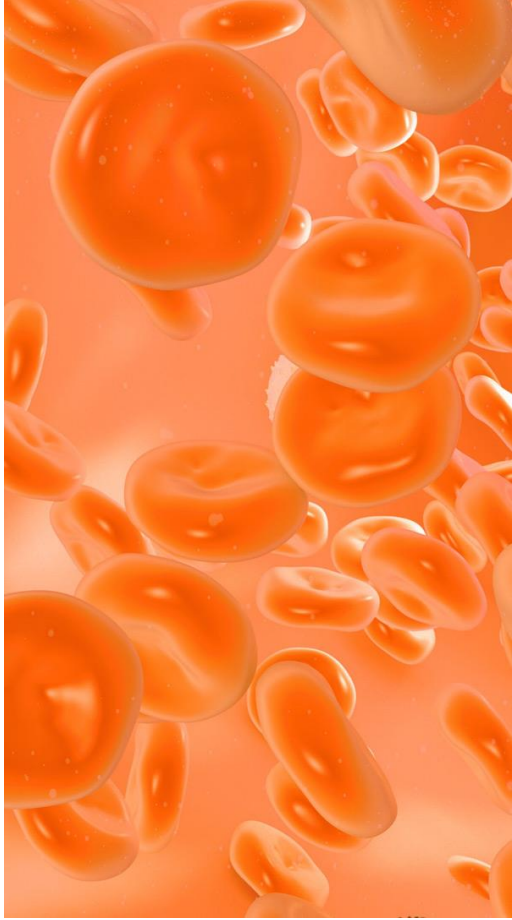
- Favourable editorial in same issue

Strong final top-line data from HORIZON announced March 26th

- ORR of 30% in ITT population
- ORR of 26% in triple-class refractory RRMM patients

NDA submission for triple-class refractory MM on track

- Pre-NDA meeting held with the FDA in December confirming plans to submit on all 157 patient included in the study
- Application for accelerated approval in triple-class refractory MM on track for Q2-20



Editorial in Lancet Haematology regarding melflufen

Is there a role for new drugs with alkylating properties in multiple myeloma?

Multiple myeloma, a complex disease originating in plasma cells, was primarily treated with melphalan until the last years of the 20th century. Advances in knowledge of the biology of the disease have led to the introduction of new drugs, and its transition of new drugs from the relapse setting to first-line treatment has been fast and as a result, most patients with multiple myeloma will receive proteasome inhibitors

intravenously every 4 weeks in combination with weekly dexamethasone can lead to clinical improvement (overall response rate was 31% [14 of 45 patients; 95% CI 18–47]; median progression-free survival was 5·7 months [95% CI 3·7–9·2]; and overall survival was 20·7 months [11·8 to not reached]). The most common toxicities were haematological toxicity and grade 3–4 thrombocytopenia and neutropenia

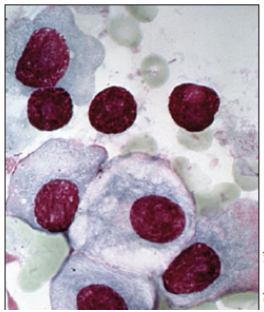


Photo Library

Source: Lancet Haematology March 23rd 2020.

Final HORIZON data in triple-class refractory RRMM

Independent Review Committee (IRC) data

Primary End-Point	Investigator Ass. Data Jan 14th	IRC Data Jan14th	Incl. unconfirmed Responses Jan 14th
Overall Response Rate (ORR) – ITT n=157	29%	30%	31% (inv. and IRC)
ORR – 3x RRMM n=119	26%	26%	27% (inv. and IRC)
ORR – EMD n=55	24%	27%	NA

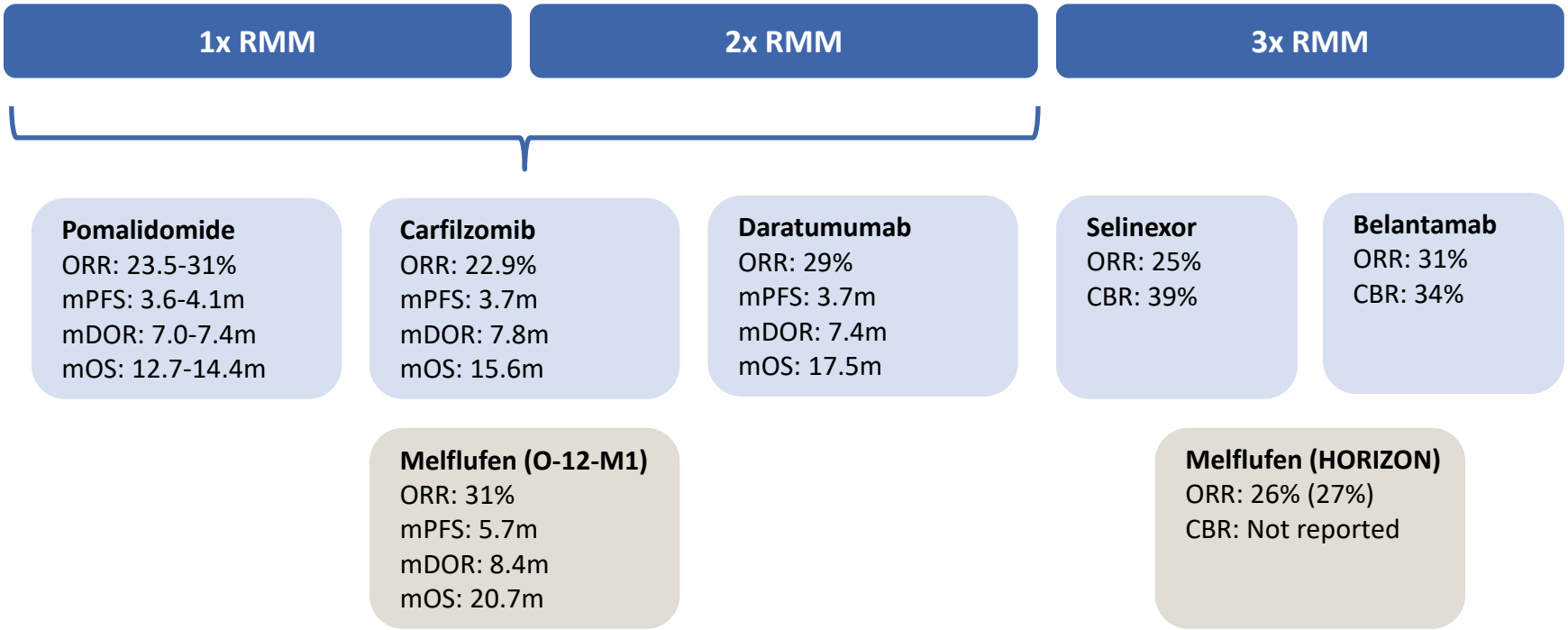
Note: Two unconfirmed responses on January 14th have later been confirmed.

Safety profile similar to the profile reported at ASH 2019, i.e. haematological toxicities were common but manageable – non-haematological toxicities were infrequent

Final data from the HORIZON study. Data in triple-class refractory patients.

Melflufen has shown excellent efficacy and tolerability

Single-agent +/- steroid – cross-study comparison



Melflufen triple-class RRMM data highly competitive

HORIZON

	Melflufen Interim data from ASH except ORR	Xpovio Karyopharm US approval July 2019	Belantamab GSK In filing
Number of patients studied	93	122	97
Overall Response/Clinical Benefit Rate	26%*/37%	25%/39%	31%/34%
Duration of response	7.5 months	4.4 months	NR (≈7-8months)
Progression-free survival	4.0 months	3.7 months	2.9 months
Overall survival	11.3 months	8.0 months	NR (≈10months)
Share of patients with EMD	34%	22%	23%
Serious Adverse Event Rate	51%	58%	36% (excl. ocular tox.)
Non-hematologic toxicity (grade 3/4) reported in >5% of patients	Pneumonia 8.4%	Fatigue 25.2% Hyponatremia 20.3% Nausea 9.8% Pneumonia 8.9% Diarrhea 7.3% Sepsis 5.7% Hypokalemia 5.7% Mental status 5.7% General det. 5.7%	Keratopathy/ Blurred vision 27.4% Hypercalcaemia 7.4% Pneumonia/ Lung infections 6.3%

* ORR number is final ORR, all other melflufen data from Interim presentation at ASH, ORR was 24% at ASH

Positive HORIZON read: Submission and US commercialization of melflufen on track – key focus 2020

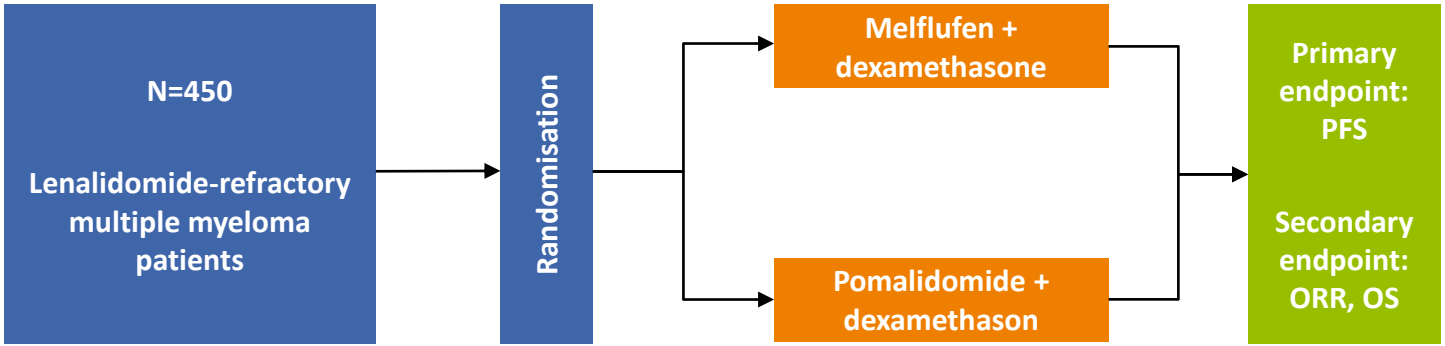
- Submission is on track for end of Q2 2020
- Early Access Program in the US to be launched as soon as feasible in RRMM patients

  Patient population without treatment options but COVID-19 makes environment challenging
- US Commercialization build-up

  The commercial organization will be built up sensibly as we get clarity on FDA timelines and financial market conditions in light of COVID-19 pandemic

Data to date provide high conviction for success in ongoing phase 3 study OCEAN

423 of 450 patients recruited as of March 20, LPI expected in April, data H2-20



RRMM data from pomalidomide FDA label and O-12-M1 study

Treatment	ORR	CBR	Median PFS	Median DOR	Median OS
Melflufen + Dexamethasone	31%	49%	5.7 months	8.8 months	20.7 months
Pomalidomide + Dexamethasone	24%	NR	3.6 months	7.0 months	12.4 months

Pomalidomide shares resistance mechanism with lenalidomide

Average IMiD free period was significant in pomalidomide registration study

- Only 29% received lenalidomide as last treatment

Lenalidomide used more aggressively today

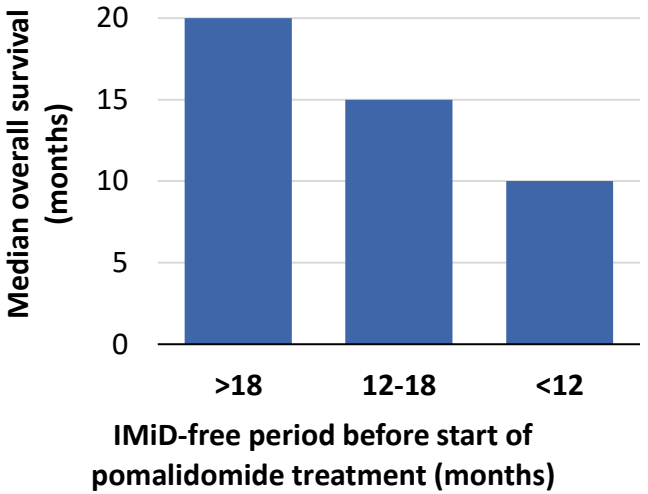
- Median maintenance duration 24 months instead of 10 months

In OCEAN all patients have failed on lenalidomide within 18 months

- Vast majority has lenalidomide as last treatment

No assumptions have been made in OCEAN power calculation to account for increased cross resistance

Pomalidomide efficacy decreases for recent lenalidomide failures

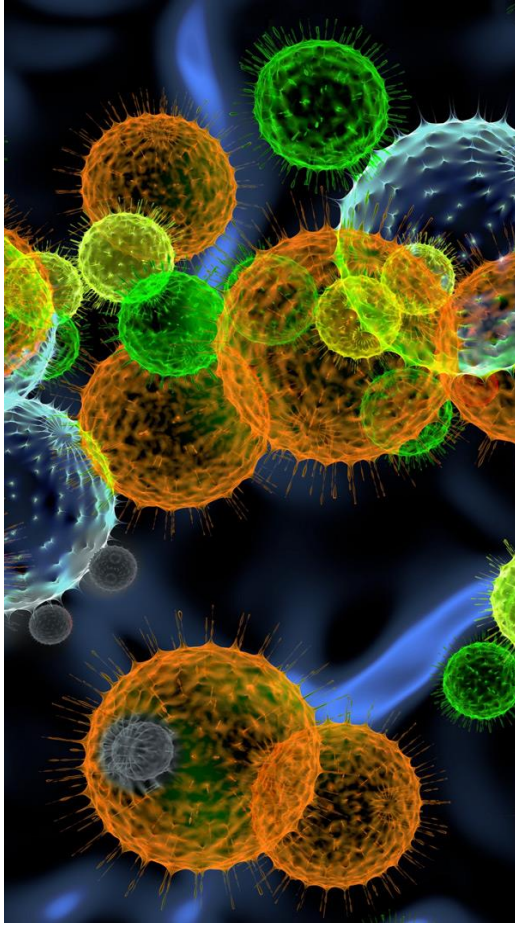


Source: Pomalidomide with Low Dose Dexamethasone Is Effective Irrespective of Primary or Secondary Resistance to Lenalidomide but the IMiD-Free Interval Is Important (Dimopoulos et. al. ASH poster 2016).

Encouraging data for melflufen in combination with daratumumab

Summary of combination data presented at ASH with daratumumab – n=33

- Median of 2 prior lines of therapy
- True RRMM population (not maintenance refractory) – 39% had disease progression while on last line of therapy and 60% high-risk cytogenetics
- **ORR of 76%** with good tolerability and deepening responses - 22 patients ongoing
- Median **PFS of 14.3 months**



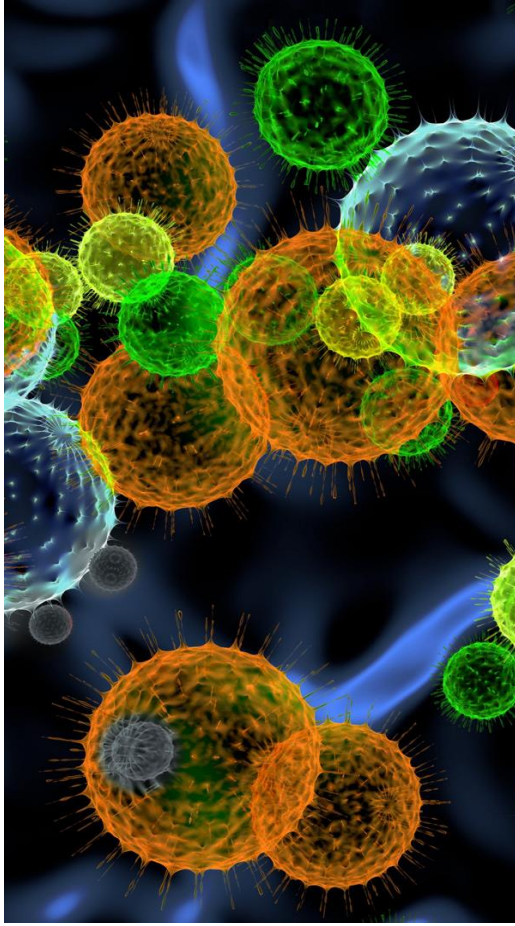
ANCHOR

Emerging data for melflufen in combination with bortezomib

Summary of combination data with bortezomib presented at ASH – n=6

- Elderly population – Median of 2.5 prior lines of therapy
- True RRMM population (not maintenance refractory) – 50% had disease progression while on last line of therapy
- 4/6 responded on therapy (**ORR 67%**) with good tolerability and deepening responses – 3 pts ongoing
- Median PFS not reached with the longest patient on treatment for 16 months

Recruitment paused due to COVID-19



ANCHOR

Combination study LIGHTHOUSE

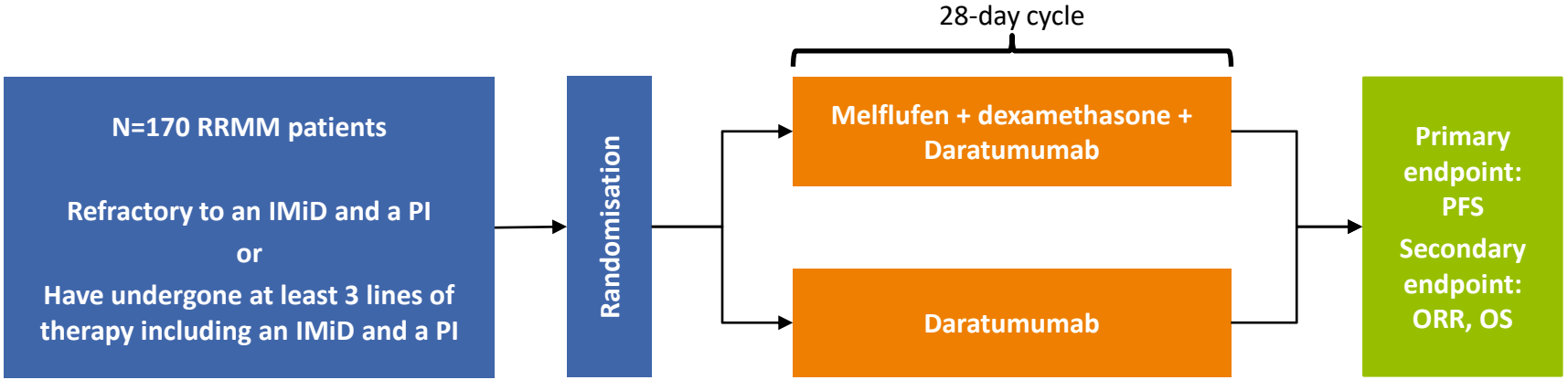
Our second confirmatory phase 3 study – initiation postponed

Second phase 3 study with melflufen in multiple myeloma

- Melflufen + daratumumab vs daratumumab randomized 2:1
- Subcutaneous version on Daratumumab

Two objectives:

- Expand market potential – extend label with melflufen in combination with daratumumab in earlier lines
- De-risk development program – add a third study that can result in market registration in the EU and US



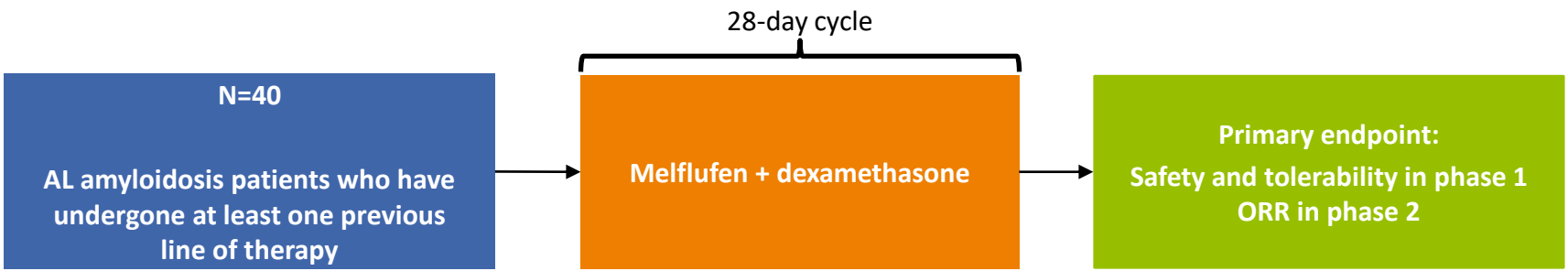
Phase 1/2 study in AL amyloidosis – recruitment paused

Similar to myeloma, AL amyloidosis is a disease of the B-cell system

- Antibody light-chains misfold and form deposits in multiple organs with organ dysfunction as a result
- Orphan disease - 30-45,000 patients in the USA and the EU[1)]
- Majority of patients >65 years old

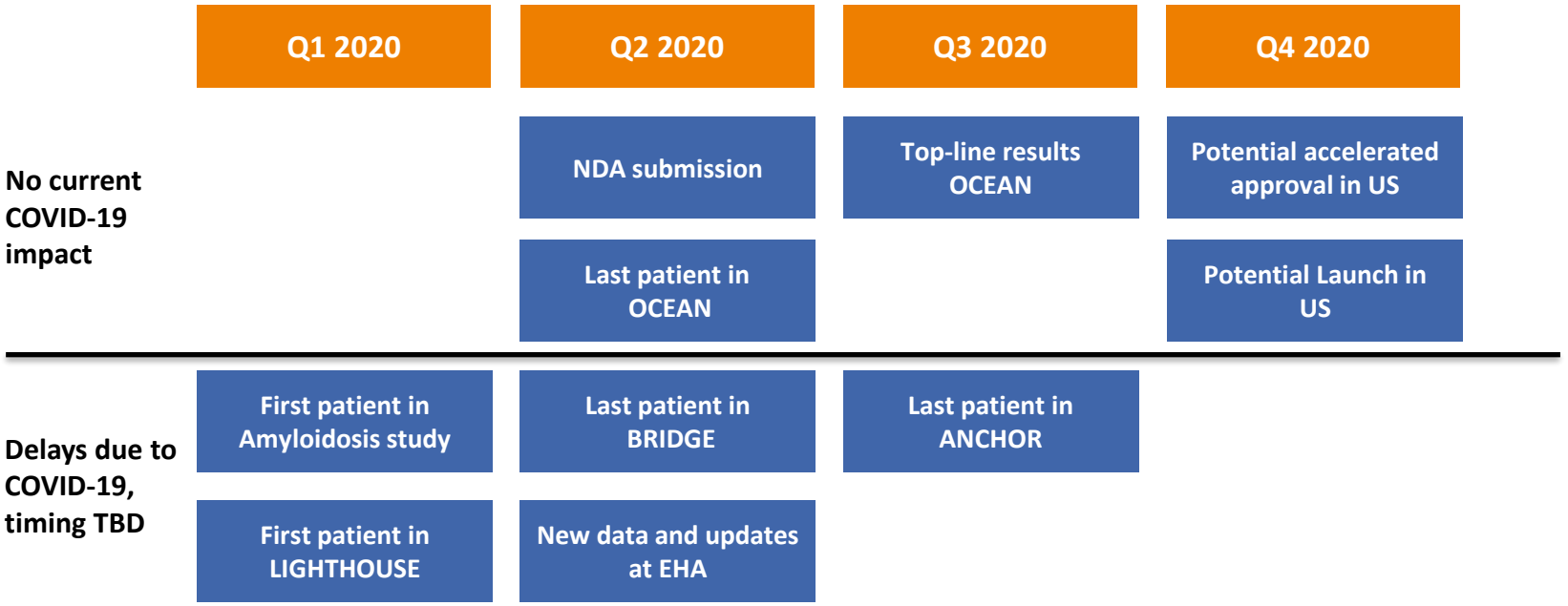
Similar drug use as for myeloma – drugs that are efficacious in myeloma are most of the time also used in AL amyloidosis

Limited treatment options with median overall survival of 1.5-2.0 years (1995-2013) with a trend towards improved survival (3.5 years for the period 2010-2013)[2)]



Source: 1) Quock et. al, Blood Advances, May 2018, 2) Weiss et. al, Blood, 2016

News flow and timelines to be updated once COVID-19 situation becomes clearer



Summary

Significant unmet needs in Multiple Myeloma

- $19 B orphan market

Melflufen has the potential to become a new treatment backbone for relapsed refractory multiple myeloma

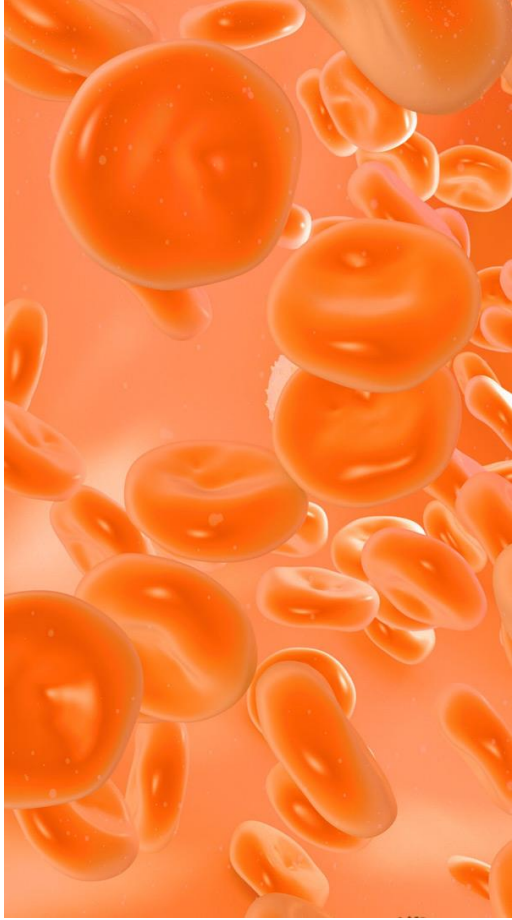
- Phase 2 study, O-12-M1, showed very strong survival data
- Both phase 2 studies, HORIZON and ANCHOR show strong overall response (ORR) data and competitive profile for progression-free survival (PFS)
- Generally well tolerated giving patients good quality of life

Late stage development program with multiple ways to get approval

- Submission for accelerated approval for triple-class refractory patients in the US on track for Q2-2020 based on HORIZON data
- Phase 3 study OCEAN expected to be fully enrolled Q1 2020
- Additional Phase 3 study, LIGHTHOUSE will start in the coming months

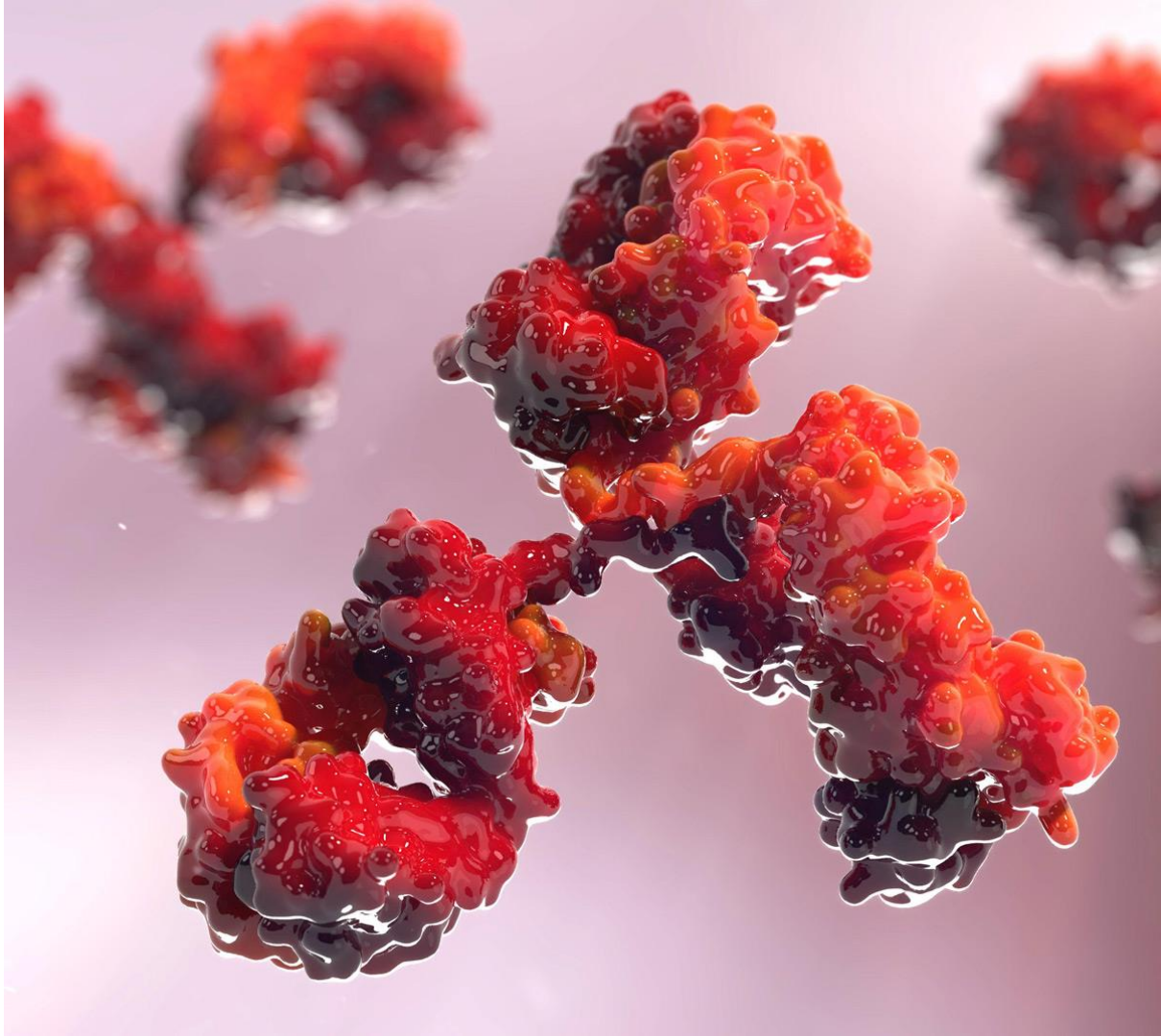
Strong financial position

- Cash position: SEK 926 M ($ 95 M) as of December 31

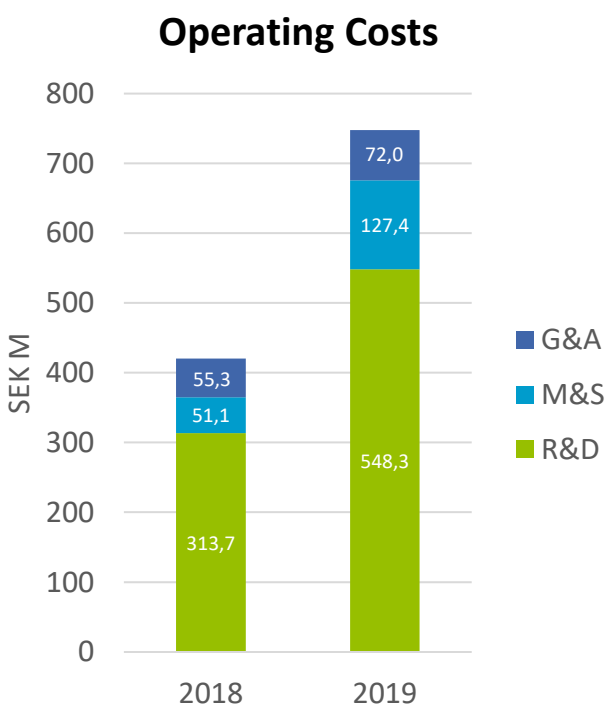


Thank you for your attention!

oncopeptides



Financial results for the period Jan – Dec 2019

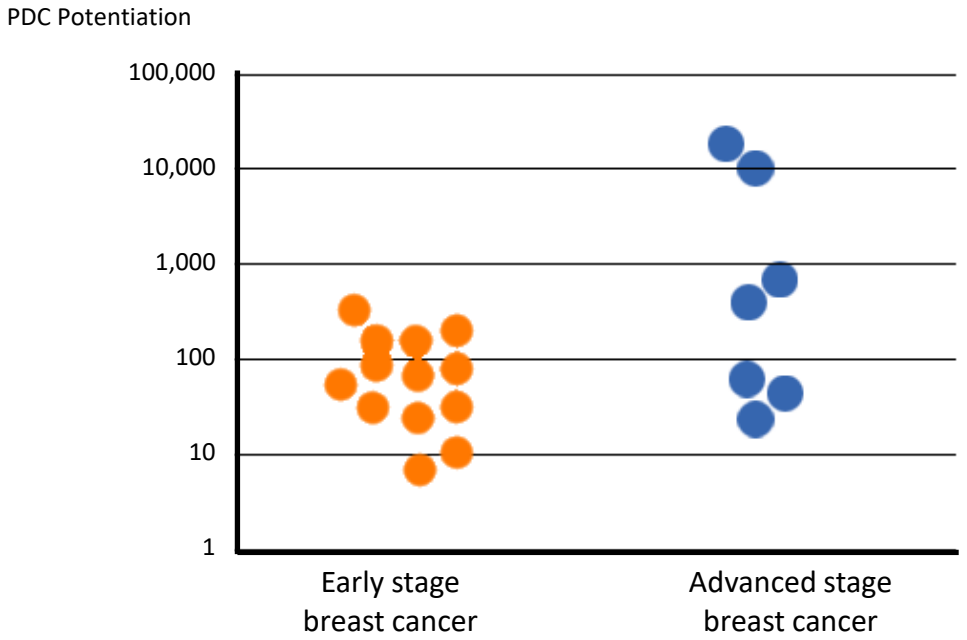


- Operating loss increased to SEK 739.4 M (loss:411.0)
  - R&D increase primarily due to increase in Clinical & drug supply: SEK 439.4 M (260.3)
    - OCEAN costs SEK 211.8 M (132.1)
    - HORIZON costs SEK 70.9 M (28.5)
    - ANCHOR costs SEK 44.6 M (25.9)
  - Build-up of commercial and medical relations explains increase in M&S costs
- Operating costs include non-cash costs related to incentive programs
  - SEK 37.8 M (54.1) for the year
- Cash flow from operating activities neg. SEK 690.6 M (neg. 333.7)
- Cash position was SEK 926.2 M (375.6) as of Dec 31, 2019
  - Directed share issue raised SEK 514.8 M after issue costs in January 2019
  - Second share issue raising SEK 682.9 M was completed in July

Shareholder structure as of March 31, 2020

Shareholder	Number of shares	Percent
Healthcap VI LP	11,322,400	20.43%
Industrifonden	7,420,805	13.39%
Gladiator	3,200,000	5.77%
Swedbank Robur Funds	2,746,502	4.96%
JP Morgan Bank Luxembourg S.A.	2,551,596	4.60%
Handelsbanken Funds	2,517,978	4.54%
4th AP Fund	2,402,742	4.34%
Oppenheimer Global Opportunities Fund	2,000,000	3.61%
AFA Insurance	1,507,554	2.72%
SEB-Foundation	1,200,000	2.17%
Avanza Pension	1,102,512	1.99%
AMF – Insurance and Funds	1,070,452	1.93%
Nordic and Europe health Invest AS	841,095	1.52%
2nd AP-Fund	820,717	1.48%
SEB SA	580,911	1.05%
Others	14,128,153	25.50%
Sum	**55,413,417**	**100%**

The single strongest activity is seen in high-risk and advanced stage breast cancer



Melfulfen is not currently approved in any indication and any potential label is subject to FDA review.

Indication broadening with the PDC platform will focus on AML, NHL and breast cancer (US data)

Indication	Triple Negative Breast Cancer	DLBCL	AML
Incidence (US)	28K (10% of breast cancer pts)	22K-29K pts (30%-40% of NHL pts)	21K
5-year survival	around 75%	30%-90% Based on cytogenetics	around 25%-30%
Standard of care	Surgery, RT, chemotherapy, PARP inhibitors, PD-(L)1 inhibitors	Relapsed: bendamustine, lenalidomide, platinum combos	Relapsed: novel therapies such as venetoclax
Initial development focus - PDC platform	Improvement of efficacy in late stages and improved toxicity profiles	Efficacy in high risk DLBCL subpopulations	Improvement of survival rates
Market opportunity - development focus (US)	~10K patients	~ 7.5-10K patients	~10-15K patients

Melfulfen is not currently approved in any indication and any potential label is subject to FDA review.

O-12-M1 phase 2 study generated best overall survival data to date in late stage myeloma

	Melflufen	Daratumumab	Pomalidomide*	Carfilzomib
N	45	106	302	266
Year	2017	2016	2013	2012
Population	Refractory to last, exposed to iMID, PI and alkylator, IMiD and PI refractory	Refractory to last, ≥3 lines with IMiDs and PI, double refractory to PI and IMiD	Refractory to last, at least 2 lines with bort and len and received alklylator	>2 prior for relapsed including Bar, Len or thal, alk or anthra alone or in combo
Time from diag.	5.0 years	4.8 years	5.3 years	5.4 years
High risk Cytog.	44%	19%	~30%	28%
Number of lines	4, 78% ≥3 lines	5, 82% ≥3 lines	5, 94 % ≥2 lines	82% ≥4 lines
Refract. to last	87%	97%	100.0%	94.0%
ORR	31.1%	29.2%	23.5%	23.7%
ORR high risk	25%	20%	–	29.6%
Med. duration treat	3.7 months	-	Progressive Disease or Unacceptable Toxicity	3.0 months
Med. duration response	8.4 months	7.4 months	7.0 months	7.8 months
Median PFS	5.7 months (11.7 in ≥PR)	3.7 months	3.6 months	3.7 months
Median OS	20.7 months	17.5 months	12.4 months	15.6 months

Source: Richardson PG *et al.,* ASH 2017; Usmani SZ *et al.,* 2016: Miguel JS *et al.,* 2013; Siegel DS *et al.,* 2012

* = source FDA label

Data from HORIZON presented at ASH 2019

HORIZON

Baseline Characteristics[a]	N-154
Age, median (range), y	64.5 (35-86)
Gender (male / female), %	56 / 44
Time since diagnosis, median (range), y	6.5 (0.7-24.6)
No. of prior lines of therapy, median (range)	5 (2-12)
ISS stage I / II / III / unknown, %	37 / 27 / 32 / 4
ECOG PS 0 / 1 / 2, %	25 / 60 / 15
High-risk cytogenetics,[b] %	38
≥2 High-risk abnormalities, %	13
Del(17p), %	12
Extramedullary disease, %	32

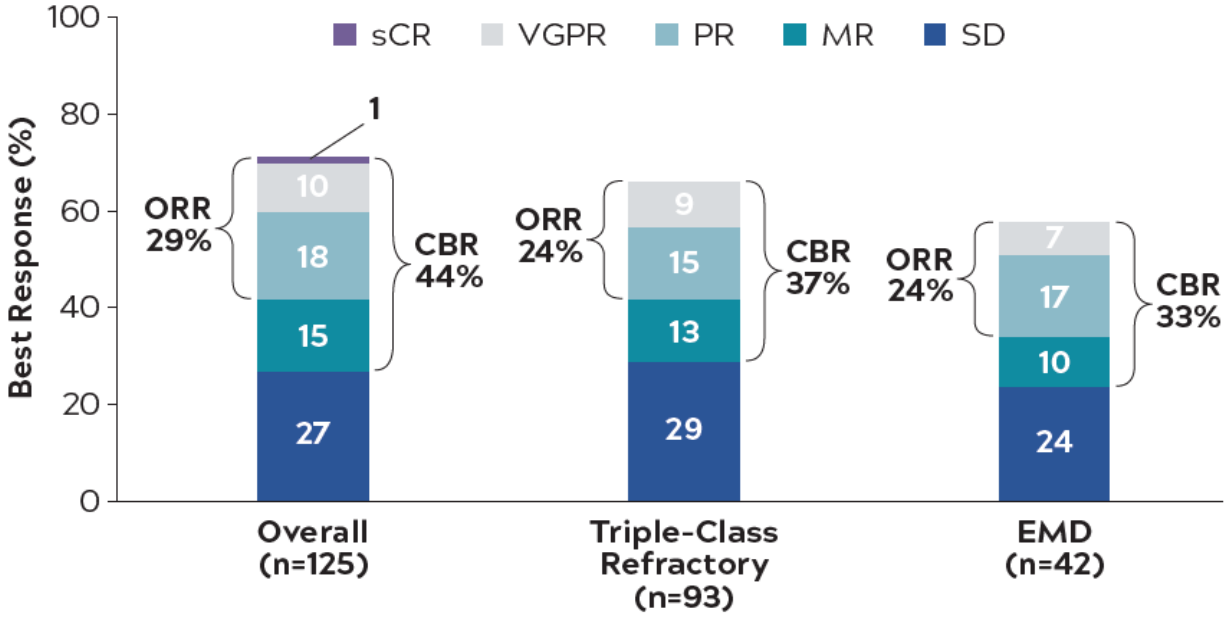
- Efficacy population (n=125): patients dosed on or before 15 May 2019 with additional follow-up of at least 20 weeks until 01 October 2019 data cutoff
- Safety population (N=154): all patients dosed on or before 01 October 2019 data cutoff

[a]Baseline is defined as the most recent assessment before administration of the first dose of study drug. [b]High-risk cytogenetics at study entry was based on fluorescence in situ hybridization defined as t(4;14), del(17/17p), t(14;16), t(14;20), gain(1q) per Sonneveld P, et al.[14] 77 patients (50%) had unknown cytogenetics.
ECOG PS, Eastern Cooperative Oncology Group performance status; ISS, International Staging System.

Source: Mateos MV, et al. ASH 2019. #1883

Promising overall response rates in both triple-class refractory patients and patients with EMD at relapse

HORIZON

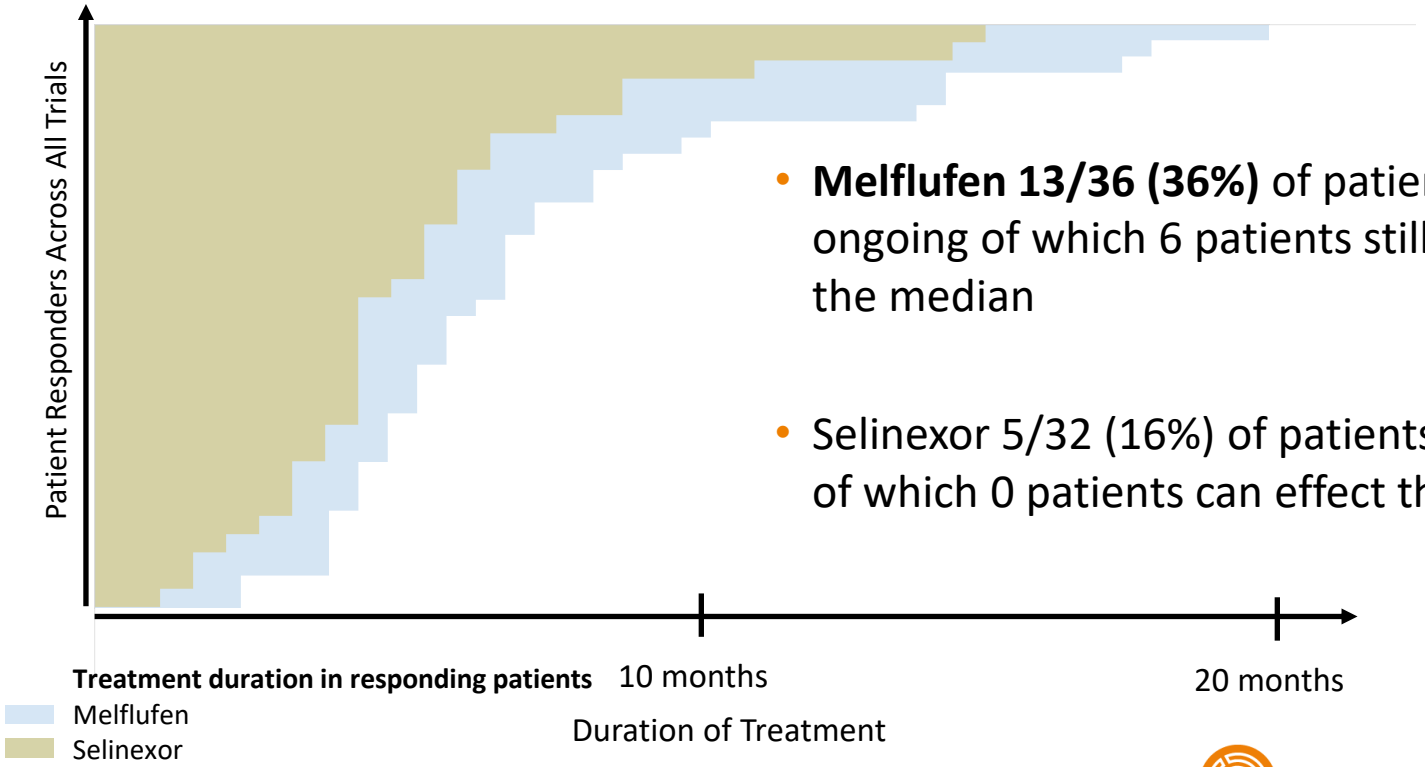


[a]Response was investigator assessed.

CBR, clinical benefit rate; EMD, extramedullary disease; IMWG, International Myeloma Working Group; MR, minimal response; ORR, overall response rate; PR, partial response; sCR, stringent complete response; SD, stable disease; VGPR, very good partial response.

Source: Mateos MV, et al. ASH 2019. #1883

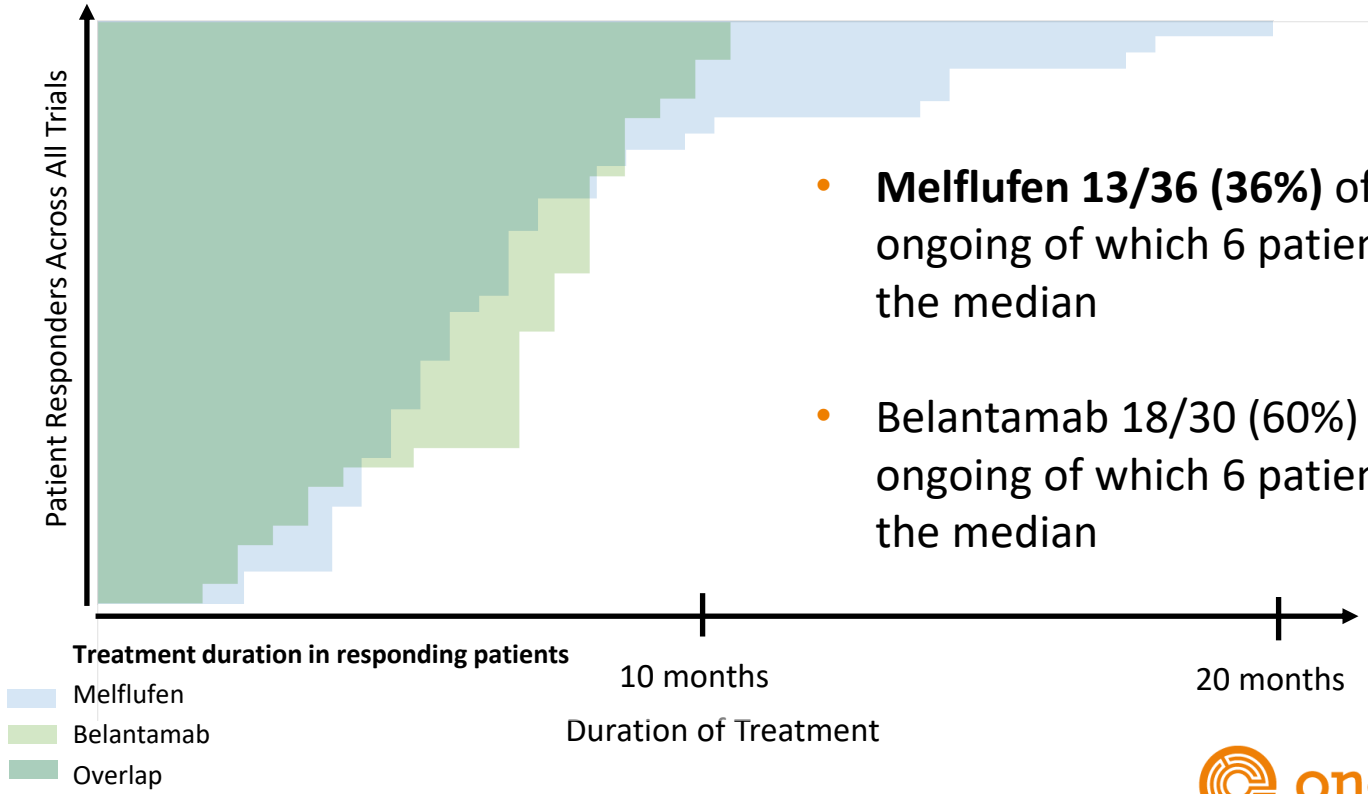
Duration of treatment: Comparison between melflufen and selinexor



- **Melflufen 13/36 (36%)** of patients still ongoing of which 6 patients still can effect the median
- Selinexor 5/32 (16%) of patients still ongoing of which 0 patients can effect the median

Source: NEJM 2019, Melflufen Data Presentation, ASH 2019

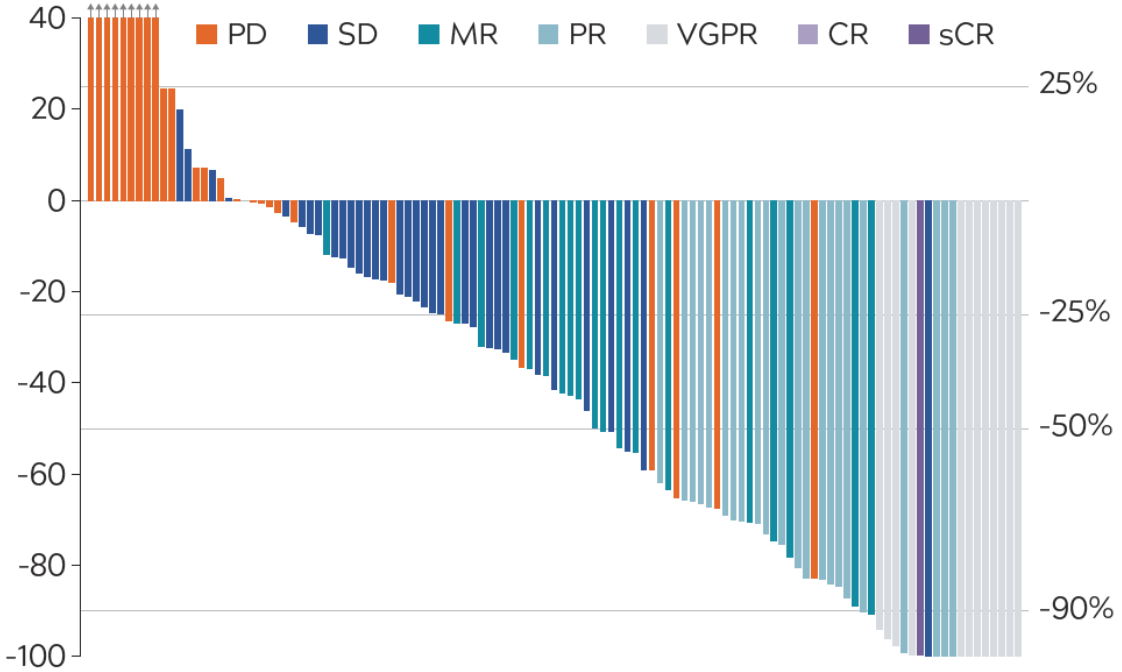
Duration of treatment: Comparison between melflufen and belantamab



- **Melflufen 13/36 (36%)** of patients still ongoing of which 6 patients still can effect the median
- Belantamab 18/30 (60%) of patients still ongoing of which 6 patients still can effect the median

Source: The Lancet 2019, Melflufen Data Presentation, ASH 2019

Disease was stabilized in 83% of patients[a]

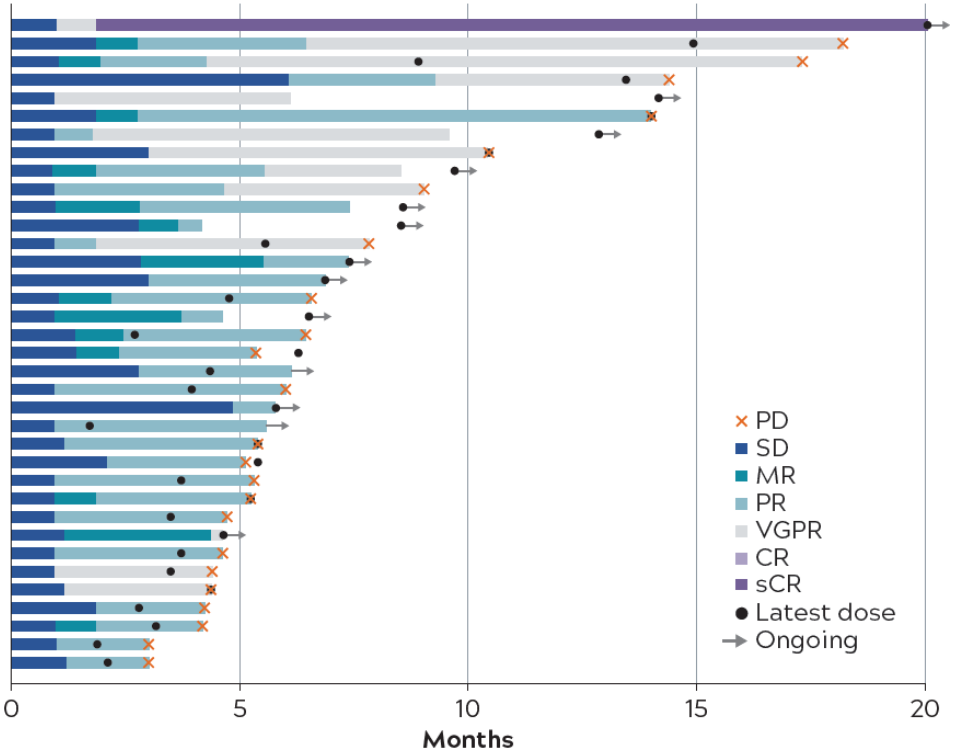


[a]In total, 10 patients had missing M-protein data.
M-protein, monoclonal protein; MR, minimal response; PD, progressive disease; PR, partial response; sCR, stringent complete response; SD, stable disease; VGPR, very good partial response.

Source: Mateos MV, et al. ASH 2019. #1883

- Overall, 83% of the patients had a reduction of M-protein despite all patients having progressing disease at study entry

Melflufen showed durable responses (n=36)a



- Median Duration of Response in triple-class refractory patients was 7.5 months

[a]The swim-lane plot is based on response assessments reported by the investigators. Gaps between the bar and latest dose indicate no response data are currently available for that time.
CR, complete response; MR, minimal response; PD, progressive disease; PR, partial response; sCR, stringent complete response; SD, stable disease; VGPR, very good partial response.

Source: Mateos MV, et al. ASH 2019. #1883

Strong patient centric safety profile

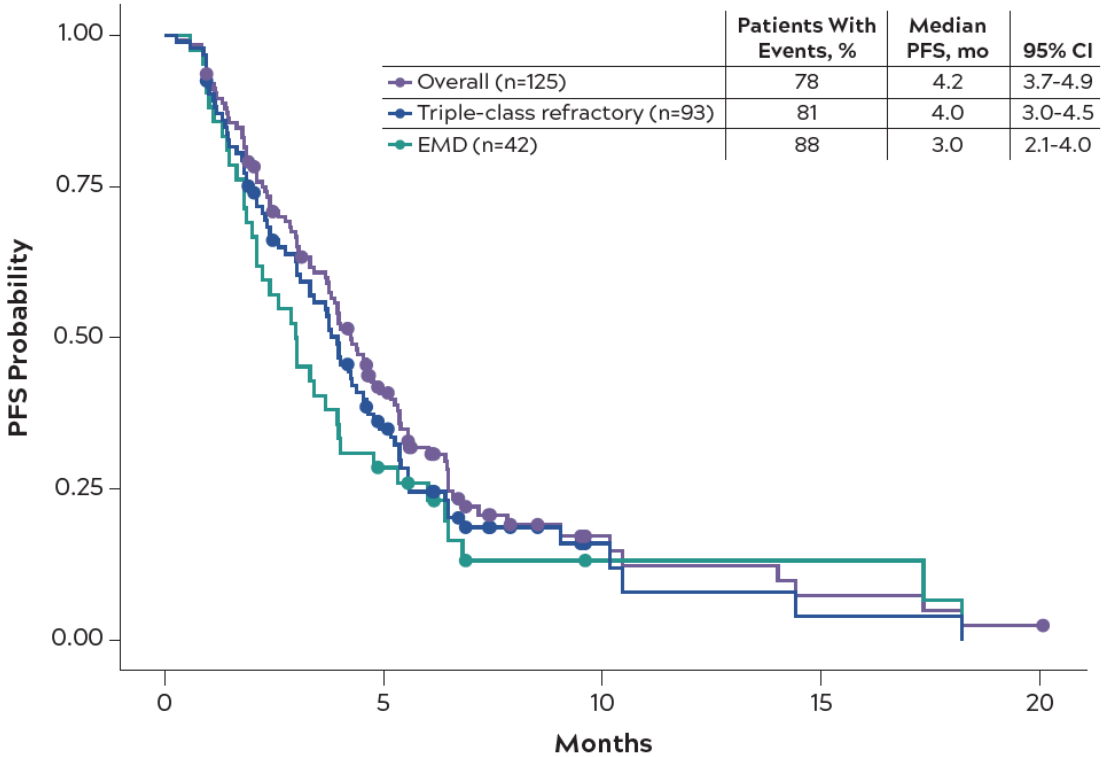
TEAE[a]	Grade 3, n (%)	Grade 4, n (%)
Anemia	56 (36)	1 (1)
Neutropenia	47 (31)	54 (35)
Thrombocytopenia	32 (21)	74 (48)
White blood cell count decreased	13 (8)	15 (10)
Pneumonia	11 (7)	2 (1)
Febrile Neutropenia	6 (4)	2 (1)
Lymphopenia	6 (4)	2 (1)
Leukopenia	4 (3)	6 (4)

[a]Grade 3 and 4 TEAEs occurring in ≥5% of patients.
TEAE, treatment-emergent adverse event.

Source: Mateos MV, et al. ASH 2019. #1883

- Absence of grade 3 and 4 TEAEs outside of the hematological system and infections and infestations
- Hematological toxicity clinically manageable
  - Only 3 patients (2%) with melflufen-related bleeding events reported as serious TEAEs
  - 71% of patients maintained the dose
- 18% of patients experienced a grade 3 or 4 infection
- 5 patients (3%) died, none was considered related to melflufen treatment

PFS Overall and in Triple-Class Refractory Patients or Patients With EMD



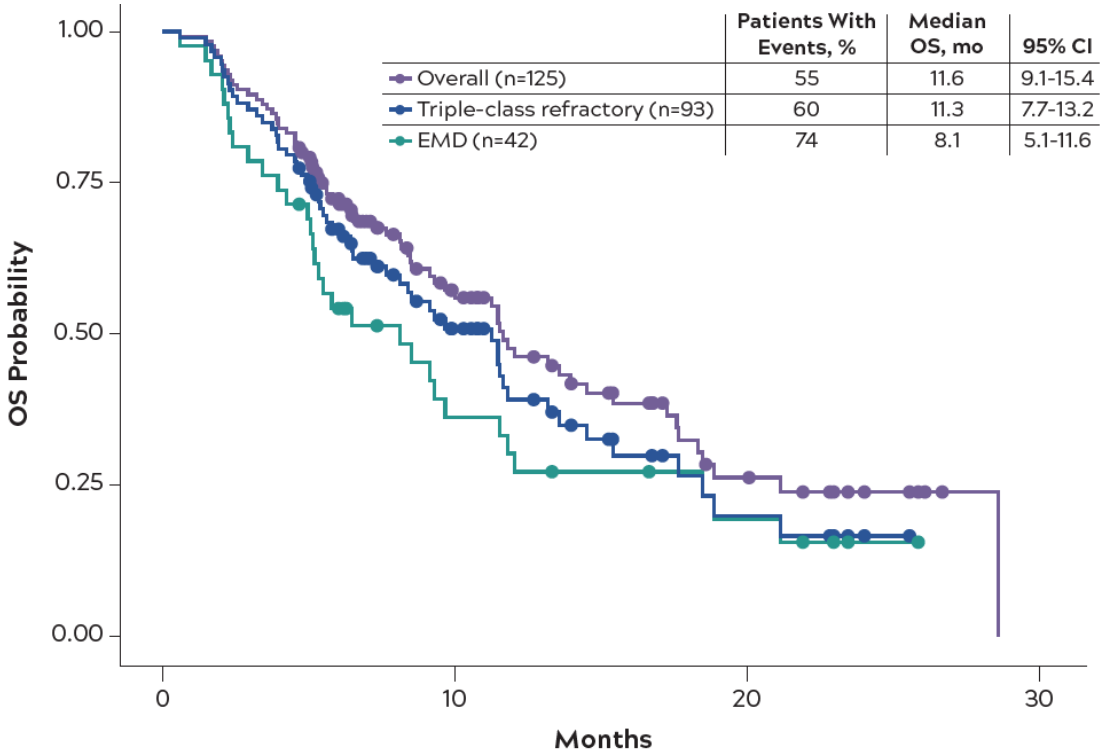
	Patients With Events, %	Median PFS, mo	95% CI
Overall (n=125)	78	4.2	3.7-4.9
Triple-class refractory (n=93)	81	4.0	3.0-4.5
EMD (n=42)	88	3.0	2.1-4.0

- Median PFS in the overall population was 4.2 months

EMD, extramedullary disease; PFS, progression-free survival.

Source: Mateos MV, et al. ASH 2019. #1883

OS Overall and in Triple-Class Refractory Patients or Patients With EMD



EMD, extramedullary disease; OS, overall survival.

Source: Mateos MV, et al. ASH 2019. #1883

- Median OS in the overall population was approximately one year

Melflufen Plus Dexamethasone in Combination With Daratumumab: Patient Characteristics

ANCHOR

Characteristics	30 mg[a] (n=6)	40 mg (n=27)
Age, median (range), y	57.0 (49-78)	66.0 (35-77)
Sex (men / women), n (%)	3 (50) / 3 (50)	19 (70) / 8 (30)
Time since diagnosis, median (range), y	3.1 (1.9-8.0)	3.8 (0.7-15.6)
No. of previous lines, median (range)	2.5 (1-3)	2.0 (1-4)
Prior ASCT / alkylator exposed, n (%)	5 (83) / 5 (83)	21 (78) / 24 (89)
Alkylator refractory, n (%)	1 (17)	3 (11)
IMiD refractory, n (%)	3 (50)	15 (56)
PI refractory, n (%)	0	13 (48)
Last-line refractory[b], n (%)	2 (33)	11 (41)
IMiD + PI refractory, n (%)	0	10 (37)
ISS at study entry, I / II / III[c], n (%)	6 (100) / 0 / 0	18 (67) / 4 (15) / 4 (15)
High-risk cytogenetics by FISH[d], n/N (%)	3/5 (60)	2/20 (60)

[a]Three patients were erroneously dosed with 30 mg of melflufen instead of the assigned 40 mg. [b]Failure to achieve at least a minimal response or progression on therapy within 60 days of treatment. [c]1 patient at the 40-mg dose level had unknown ISS. [d]High risk defined as t(4;14), t(14;16), t(14;20), del(17/17p), or gain(1q). Missing data for 1 patient at the 30-mg dose level and 7 patients at the 40-mg dose level.

ASCT, autologous stem cell transplantation; FISH, fluorescence in situ hybridization; ISS, International Staging System; PI.

Source: Ocio EM, et al. ASH 2019. #3124

- At the time of data cutoff (8 October 2019), 33 patients had been treated with melflufen (6 patients with melflufen 30 mg, 27 patients with melflufen 40 mg) plus dexamethasone in combination with daratumumab
- Baseline characteristics were as expected in patients with RRMM

Melflufen in combination with daratumumab: Response assessment

ANCHOR

- Of the 33 patients, 25 responded to treatment, with an ORR of 76% and a CBR of 79%

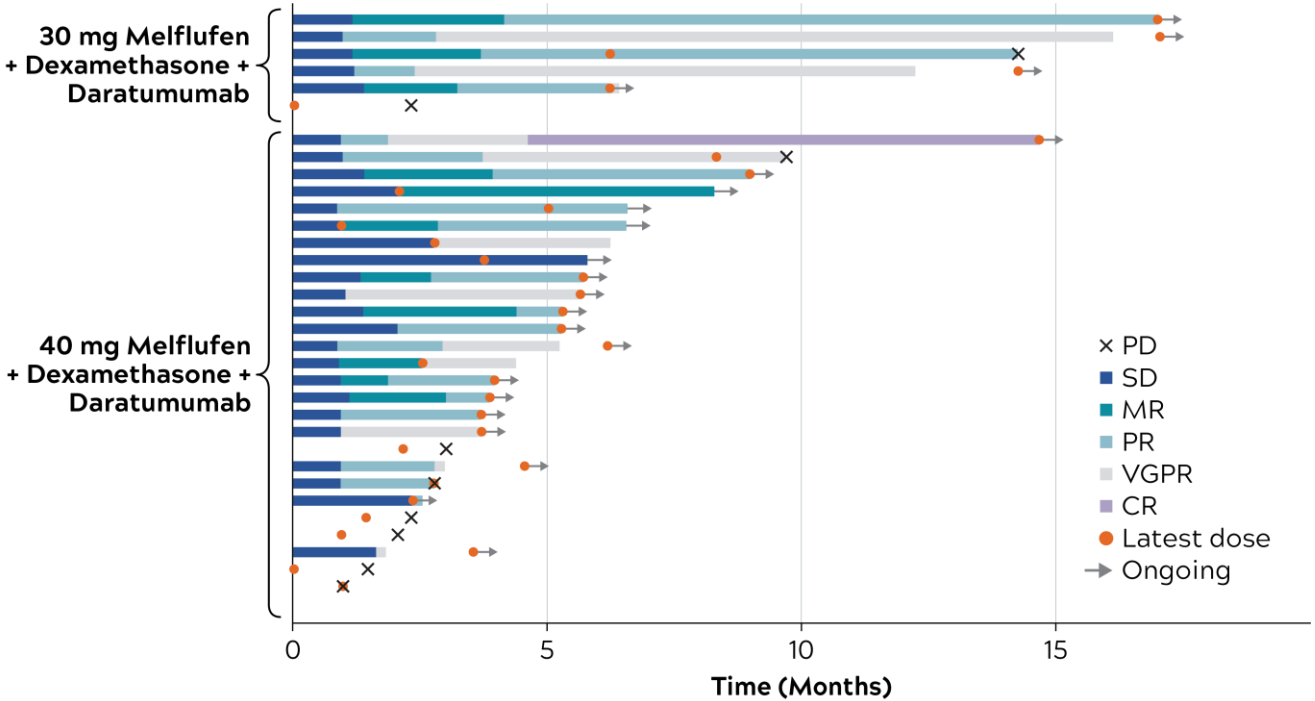
Subgroup	Patients, n							Patients, %	
	sCR	CR	VGPR	PR	MR	SD	PD	ORR	CBR
Total (n=33)	1	0	11	13	1	2	5	76	79
Melflufen 30 mg (n=6)	0	0	3[a]	2	0	0	1	83	83
Melflufen 40 mg (n=27)	1	0	8[b]	11	1	2	4	74	78

[a]Includes 1 unconfirmed VGPR.
[b]Includes 2 unconfirmed VGPRs.
CBR, clinical benefit rate; CR, complete response; MR, minor response; ORR, overall response rate; PD, progressive disease; PR, partial response; sCR, stringent CR; SD, stable disease; VGPR, very good PR.

Source: Ocio EM, et al. ASH 2019. #3124

Deepening responses, 22 of 33 patients ongoing

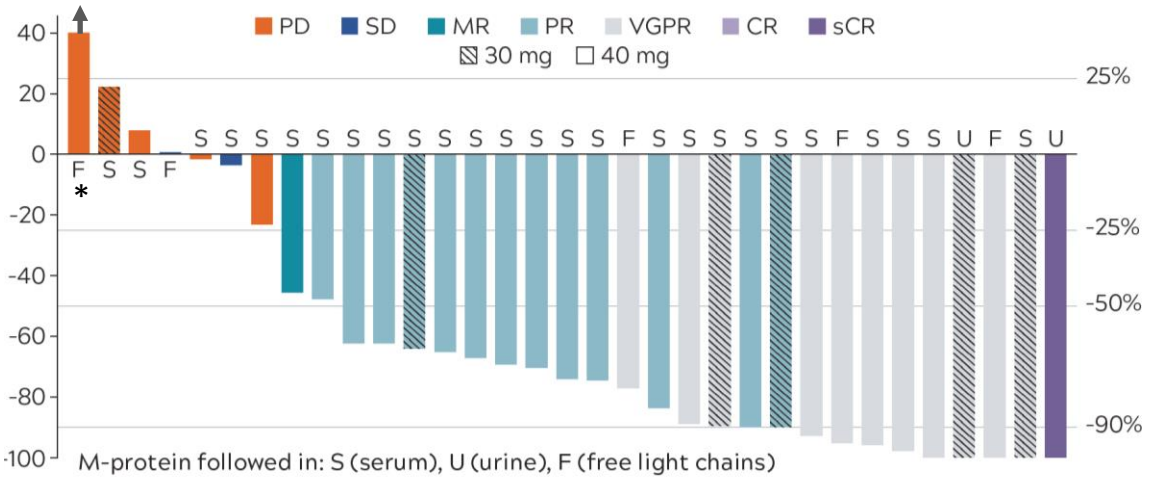
ANCHOR



[a]The swim-lane plot is based on response assessments reported by the investigators. Gaps between the bar and latest dose indicate there were no response data available for that time. CR, complete response; EoT, end of treatment; MR, minor response; PD, progressive disease; PR, partial response; SD, stable disease; VGPR, very good PR.

Source: Ocio EM, et al. ASH 2019. #3124

Melflufen Plus Dexamethasone in Combination with Daratumumab: Waterfall Plot (Best M-Protein Change)



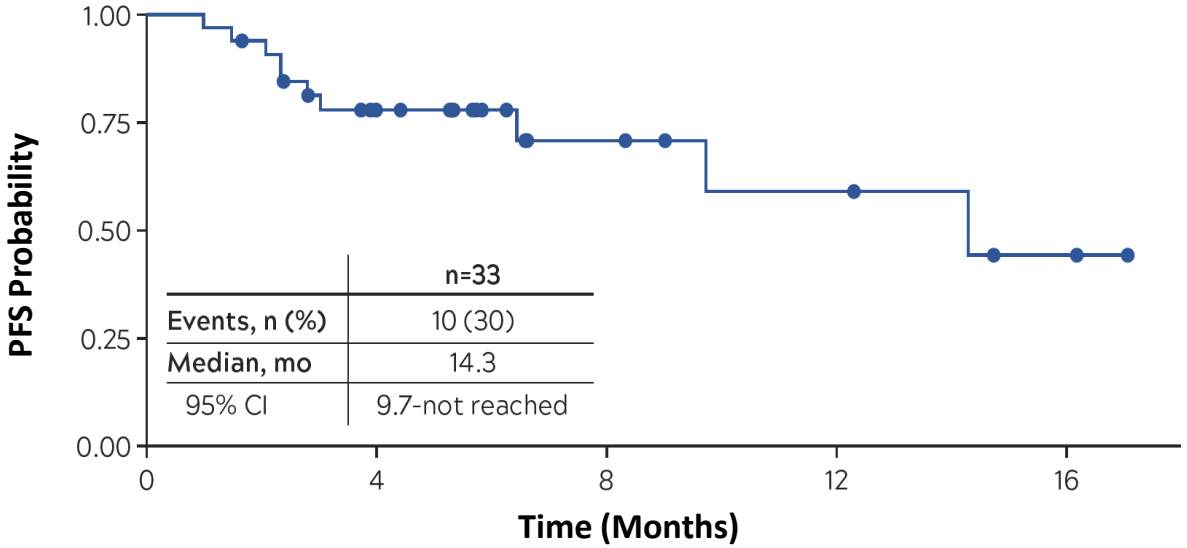
- Most patients were progression-free at data cutoff, with 10 events in 33 patients
  - Median PFS was 14.3 months (95% CI, 9.7-not reached)
  - Patients were censored on their latest progression-free observation

*Measurable disease by UPEP at baseline but not evaluated at response assessment.
CR, complete response; MR, minor response; PD, progressive disease; PR, partial response; sCR, stringent CR; SD, stable disease; UPEP, urine protein electrophoresis; VGPR, very good PR.

Source: Ocio EM, et al. ASH 2019. #3124

Emerging Progression-Free Survival of 14.3[a] months

ANCHOR



- Most patients were progression-free at data cutoff, with 10 events in 33 patients
  - Median PFS was 14.3 months (95% CI, 9.7-not reached)
  - Patients were censored on their latest progression-free observation

[a]These data are immature since 23 patients are still in PFS follow-up.
PFS, progression-free survival.

Source: Ocio EM, et al. ASH 2019. #3124

Melflufen Plus Dexamethasone in Combination with Daratumumab: Treatment-Related Grade 3/4 AEs (n=33)[a]

ANCHOR

	Treatment-Related Grade 3/4 AEs	
Preferred Term	**30 mg (n=6) Patients, n (%)**	**40 mg (n=27) Patients, n (%)**
Any Grade 3/4 AE	5 (83)	22 (81)
Thrombocytopenia[b]	3 (50)	18 (67)
Neutropenia[b]	5 (83)	15 (56)
Anemia	3 (50)	2 (7)
Febrile neutropenia	1 (17)	1 (4)

[a]Treatment-related grade 3/4 AEs reported occurred in at least 1 patient in the 40-mg cohort. Additional treatment-related grade 3/4 AEs that occurred in 1 (4%) patient each in the 40-mg cohort included pancytopenia, upper respiratory tract infection, fatigue, pyrexia, infusion-related reaction, respiratory failure and sepsis (grade 4 events both occurring in 1 patient; events later worsened to grade 5), agitation, muscular weakness, increased blood alkaline phosphatase, hypertension.
[b]Event terms include platelet count decreased and neutrophil count decreased, respectively.
AE, adverse event.

- No DLTs were observed at any dose level in the phase 1 part of the study
- The combination of melflufen, dexamethasone, and daratumumab was well tolerated, with clinically manageable grade 3/4 hematologic AEs, and the low number of nonhematologic AEs was noteworthy
- 3 Patients died, all with myeloma progression (one detected at autopsy)
  - 1 Patient had grade 5 sepsis and pneumonia while in progression (considered study treatment-related)

Source: Ocio EM, et al. ASH 2019. #3124