

# **Ygalo<sup>®</sup> - Targeted Alkylator for the Treatment of Myeloma**

**Reflections from ASH 2017 - WebCast**

**13<sup>th</sup> of December, 2017**

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# Today's Webcast



**Speaker:** Jakob Lindberg, CEO  
Oncopeptides AB

**Time:** 14:30-15:15

**Agenda:**

- Reflections from ASH
- Data highlights
- Q&A

# ASH reflections with focus on myeloma (part I)

## Strong Data Reveal from O-12-M1 and HORIZON

- Ygalo® has both pronounced efficacy of its own and facilitates for further treatment following progression
- Best OS data to date compared to trials with other compounds in late-stage RRMM patients
- Best PFS data to date compared to trials with other broad spectrum agents in late-stage RRMM patients
- Once monthly – 30min i.v. infusion - with almost clean tolerability profile outside of myelosuppression differentiates Ygalo® compared to competition in terms of tolerability and ease of use (in addition to the strong efficacy)

## BCMA Verified as a Clinically Relevant Myeloma Target

- BCMA showed clinically relevant data in myeloma with antibody and CAR-T based therapies
- BCMA based therapies do not cure myeloma patients but showed a good efficacy signal in myeloma
- In particular, GSK's anti-BCMA ADCC had an efficacy signal in-line with the CAR-T data but without all the limitations and complexities that come with CAR-T based therapies

# ASH reflections with focus on myeloma (part II)

## Daratumumab Continues to Provide Rich Amounts of Data

- Daratumumab continues to provide rich amounts of data in myeloma
- Janssen/JnJ needs to successfully develop the subcutaneous daratumumab formulation (in collaboration with Halozyme) to fully capture the potential of the antibody

## Other Myeloma News

- Selinexor from Karyopharm continues to show combination data with other compounds. Karyopharm seemingly pursues a combination strategy instead of a single-agent strategy in myeloma
- More data with venetoclax together with bortezomib in patients with t(11:14)
- Minimum Residual Disease (MRD) continues to show importance in CR patients to show
- Real-world data continues to be behind clinical trial data for a lot of compounds in myeloma. In the real world setting, single agent (w or w/o steroid) is the most common treatment, patients are older and more frail, and ease of use and tolerability are more important factors than in the clinical trial setting

# ASH reflections with focus on myeloma (part III)

## CAR-T News in General

- CD19 based CAR-T therapies continues to show good data – in particular in children and young adults with ALL. Some patients show no disease remission 2 years after treatment with an historical survival time of 3-6 months
- The complexity of giving CAR-T based therapies both in terms of manufacturing and care was highlighted in several sessions with most hospitals at a loss how to be able to offer the therapy
- The cost of CAR-T based therapy, where in a US setting the drug in itself costs around 400,000 USD with an additional almost double that amount in in-hospital care costs, shatters previous treatment cost records. Currently, in the US, a hospital takes a loss of 70,000 USD just on the drug alone. In-hospital care costs then have zero remaining reimbursement, resulting in a total loss of 500-800,000 USD per patient. Hospitals have started to ask patients to pay the delta out-of-pocket prior to treatment initiation to deliver care



## Melflufen is a targeted alkylator challenging the treatment paradigm in RRMM

**Where:** Omni Atlanta at CNN Center (Pecan Room/Foyer) 100 CNN Center, Atlanta, GA 30303

**When:** Sunday, December 10, 2017  
Reception 8:00 – 8:30 PM and Scientific Program 8:30 – 10:00 PM

By Invitation Only

**Speakers:** **O-12-M1 - Long-term follow-up from phase-2 data and reflections around the role of melflufen in multiple myeloma**

**Paul Richardson, MD**

*RJ Corman Professor of Medicine Harvard Medical School, Clinical Program Leader and Director of Clinical Research Jerome Lipper Multiple Myeloma Center Dana-Farber Cancer Institute Boston, Massachusetts*

**Horizon - Initial activity of melflufen after pomalidamide and daratumumab failure**

**María-Victoria Mateos, MD**

*Associate Professor of Medicine and Consultant Physician in the Hematology Department of the University Hospital of Salamanca, Salamanca, Spain*

**Host:** **Bengt Gustavsson** Dr Med Sci, MSc Pharm,  
*Medical Relations, Oncopeptides AB, Stockholm, Sweden*



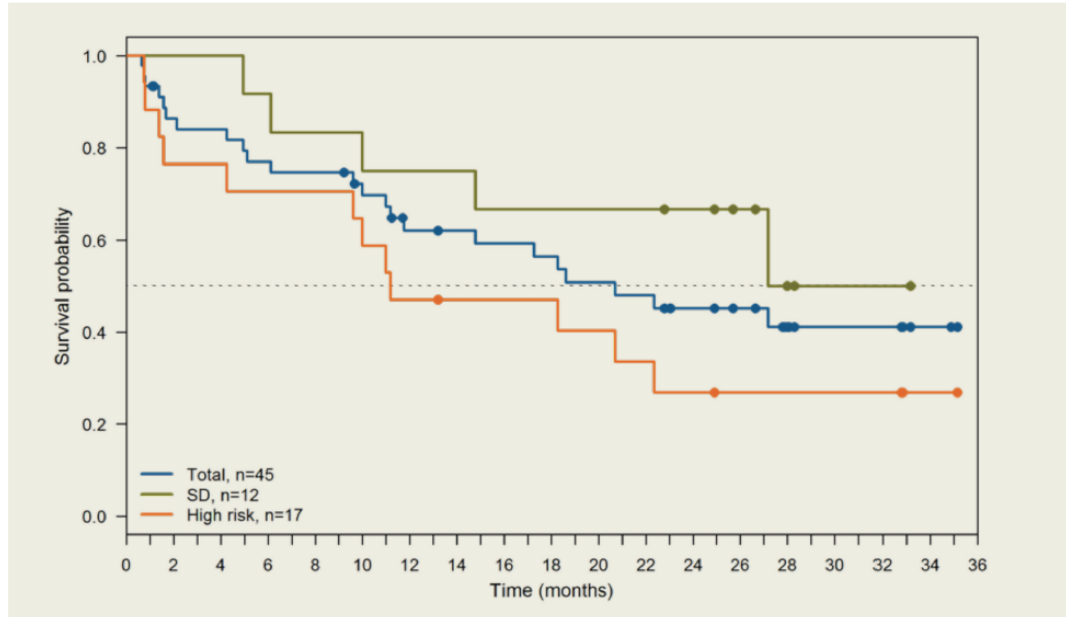
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# DATA HIGHLIGHTS

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# Best-in-class survival data from Ygalo® in phase II in late-stage RRMM

Figure 2. Overall survival



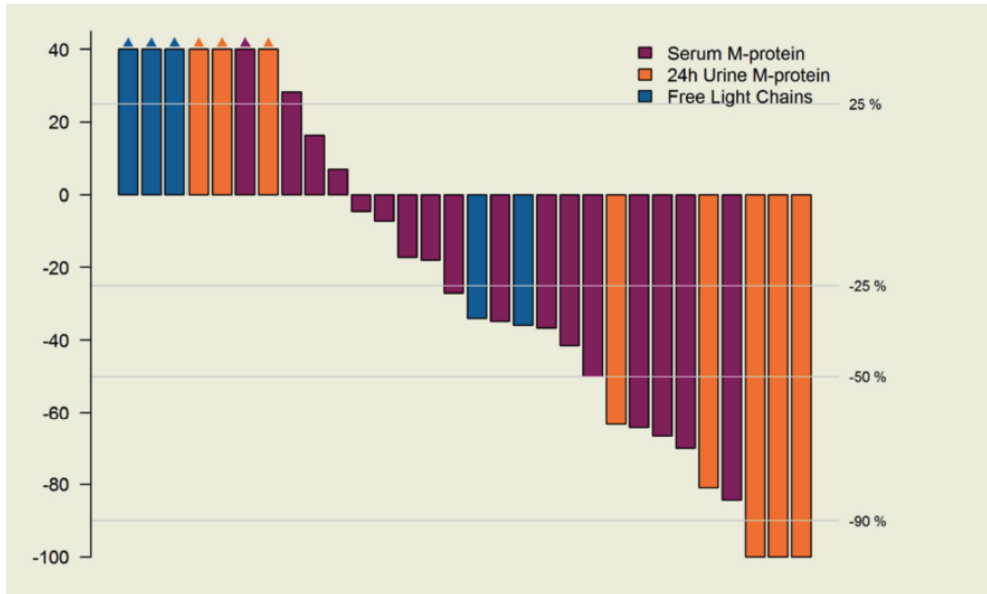
- Ygalo® has both pronounced efficacy of its own and facilitates for further treatment following progression
- Best-in-class survival data in late-stage RRMM
- Best PFS to date for a broad-spectrum agent in late-stage RRMM
- Tolerability profile very favorable with patients experiencing comparatively few side-effects that are detrimental to QoL (which in a palliative care setting with elderly patients is key)
- Confidence in reaching end-point in OCEAN has increased

# Promising efficacy signal in HORIZON with excellent tolerability in patients with no or few remaining treatment options

**Table 5. Overall response rate (N=30)**

N	PD	SD	MR	PR	VGPR	ORR	CBR
Adjusted ITT, n (%)	11 (37)	9 (30)	2 (7)	6 (20)	2 (7)	26.7%	33.3%

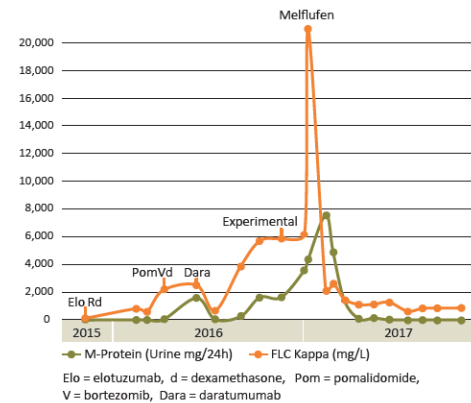
**Figure 1. Waterfall plot (N=30)**



**Table 6. Treatment-related G3/4 AEs occurring in ≥ 5% of the patients (N=38)**

	GRADE 3 OR 4, n (%)	GRADE 4, n (%)
Any treatment-related AE	22 (58)	15 (39)
Blood and lymphatic system disorders	20 (53)	14 (37)
Thrombocytopenia	17 (45)	12 (32)
Neutropenia	15 (39)	9 (24)
Leukopenia	3 (8)	3 (8)
Anemia	8 (21)	0
Lymphopenia	3 (8)	0
Hemolytic anemia	2 (5)	0

**Figure 2. Patient case study**



42-year old man with ISS stage 3, MM diagnosed 2007. No detectable serum M-protein. Nine prior lines of therapy including ASCT X 2 and Allo-SCT. Refractory to R, Elo, V, Pom, Dara and an experimental PD to the last 4 lines of therapy.

Following 5 cycles of melflufen, the urinary M-protein was undetectable (Figure 2). The patient has received 9 cycles of melflufen, achieved VGPR and is ongoing as of Nov 2017.

# BCMA clinically verified as a good target in myeloma

Study	NIH	Celgene/BB	Celgene/BB	Celgene/BB	Uni. Of Pen	Soochow Uni.	GSK
Source	ASH 2016	ASH 2016	ASCO 2017	ASH 2017	ASH 2017	ASH 2017	ASH 2017
Target	BCMA (CAR-T)	BCMA (CAR-T)	BCMA (CAR-T)	BCMA (CAR-T)	BCMA (CAR-T)	BCMA + CD19 (dual CAR-Ts)	BCMA ADCC
Patient Selection (% of total myeloma population)	50%	20-25%	20-25%	20-25%	50%	20-25%	100%
# of patients	16	6*	20*	20*	24	10	10
ORR	50%	100%	75%	85%	47%	80%	60%
Median PFS	<4 months	NA	4+ months	Est. at 11-12 months**	Roughly 4 months	Roughly 4 months	8 months

**Comment:** BCMA clinically verified as a good target in myeloma (still no cure). Currently no positive differentiating factor in terms of efficacy for CAR-T over antibody based BCMA therapies, meaning that BCMA CAR-Ts will struggle due to added cost, toxicity and overall complexity

\* Only what Celgene/BB considers as the right doses used in the comparison, i.e. the dose range that they state will be used in future studies

\*\* Estimated – median not reached after 9 months (61% without progression in ITT population)

# CAR-T shows promise in some indications but the challenges are immense

What CAR-T therapies reaches the efficacy threshold to be a valid treatment alternative?



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**Efficacy**



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**Short-term toxicity sometimes severe/life threatening**

**Long-term toxicity unknown (patient has CAR-Ts in their system for life)**

**Complex procedure for the hospital – requires several special resources/departments/certifications**

**Very costly (product and associated hospital care – current average estimated US cost in total around 1 MUSD per patient)**

## WebCast Summary

- Strong final data – often best-in-class – in the phase II clinical study O-12-M1 with Ygalo® in late-stage RRMM. Ygalo® has both pronounced efficacy of its own and facilitates for further treatment following progression. Ygalo® is easy to use with excellent tolerability profile
- HORIZON showed good activity in myeloma patients with no or few remaining treatment options with excellent tolerability profile in these patients with very small bone-marrow reserves
- The largest update at ASH is that BCMA is now a clinically verified good target for the treatment of myeloma. Both CAR-T and antibody based data was shown with little to no differentiating factors between the two (meaning that the BCMA directed antibodies have a clear lead)
- Daratumumab continued to show clinical data across several clinical trials. Selinexor continued to show strong combination data. Venetoclax continued to show data in patients with specific high-risk cytogenetics
- CAR-T was discussed at length with enthusiasm around the concept. CD19 directed CAR-T therapy in children and young adults with ALL stands out as the strongest showing of efficacy. Serious concerns are currently raised regarding toxicity, organizational requirements at the hospitals delivering the care and costs associated with CAR-T treatment

# Q&A

A microscopic view of a cluster of cells, with one cell in the center being more prominent and showing detailed surface texture. The overall color is a warm, brownish-orange.

**Thank you for your time**