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Q4 2016
Financial Results
& Business Update

March 9, 2017

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Agenda

Prepared Remarks

- **Corporate Update and ZGN-1061 Differentiation**
  - Tom Hughes, Ph.D., President and Chief Executive Officer
- **Clinical Update**
  - Dennis Kim, M.D., Chief Medical Officer
- **Financial Results**
  - Patty Allen, Chief Financial Officer

Corporate Update and ZGN-1061 Differentiation

Tom Hughes, Ph.D., President and Chief Executive Officer

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Leveraging Experience to Advance Development of Second-Generation MetAP2 Inhibitors

Recent Progress

- Phase 1 clinical trial for ZGN-1061 nearing completion
- Advanced understanding of ZGN-1061's key differentiating characteristics relative to beloranib, with emphasis on drug safety

Strategic Focus for 2017

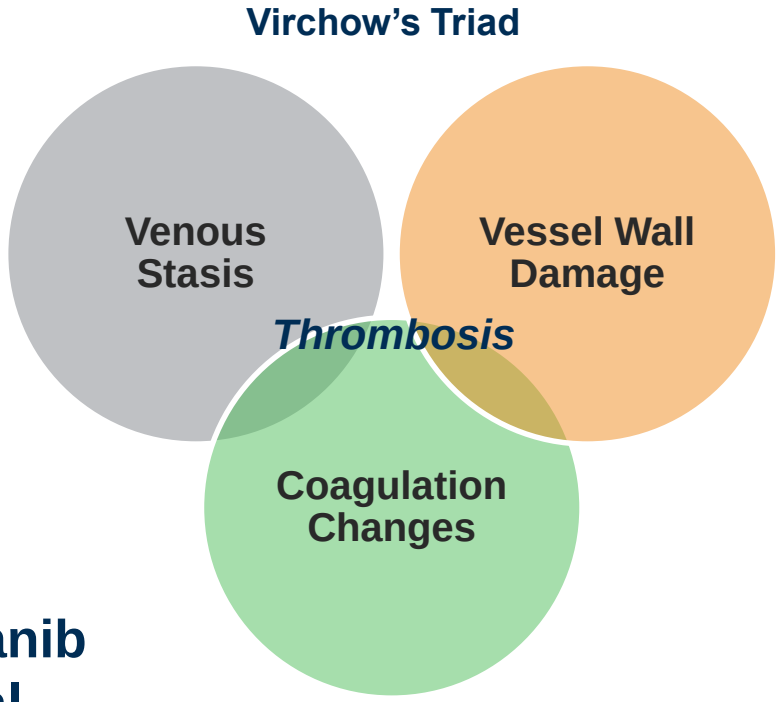
- Initiate Phase 2 clinical trial in patients with obesity and type 2 diabetes
- Further establish differentiation vs. beloranib
- Define path forward for ZGN-1061 in commercially-relevant patient populations
- Advance research activities focused on second-generation MetAP2 inhibitors

ZGN-1061: Highly Optimized, More Advanced MetAP2 Inhibitor

		Beloranib	ZGN-1061
Efficacy	**Impact on weight loss, glycemic control, CV risk factors**	Fully effective based on pre-clinical and clinical data	Fully effective based on pre-clinical data
Pre-clinical Safety	**Embryofetal development impact**	No margin	Improved margin
	Testicular function impact	Narrow margin	No impact
	Thrombosis	Narrow margin	Substantial margin
Economics	**Royalties/milestones due**	Up to $22.5M in milestones; single digit royalties	None; Wholly-owned
	Manufacturing	Complex	Simplified
	Patent life	2029-2031	2036+
Opportunity	**Markets**	Orphan indications	Prevalent metabolic indications
	Lead indication(s)	PWS, HIAO	Type 2 diabetes/obesity

Summary of Pro-thrombotic Effects of Beloranib

- No effects of beloranib effects seen on
  - Platelets or platelet aggregation
  - Neutrophil adhesion or NETs formation
  - Clotting factor levels or function
  - Blood clotting or clot lysis
- Effects of beloranib seen on vessel wall cell function
  - Endothelial proliferation slowed
  - Endothelial cell anticoagulant function

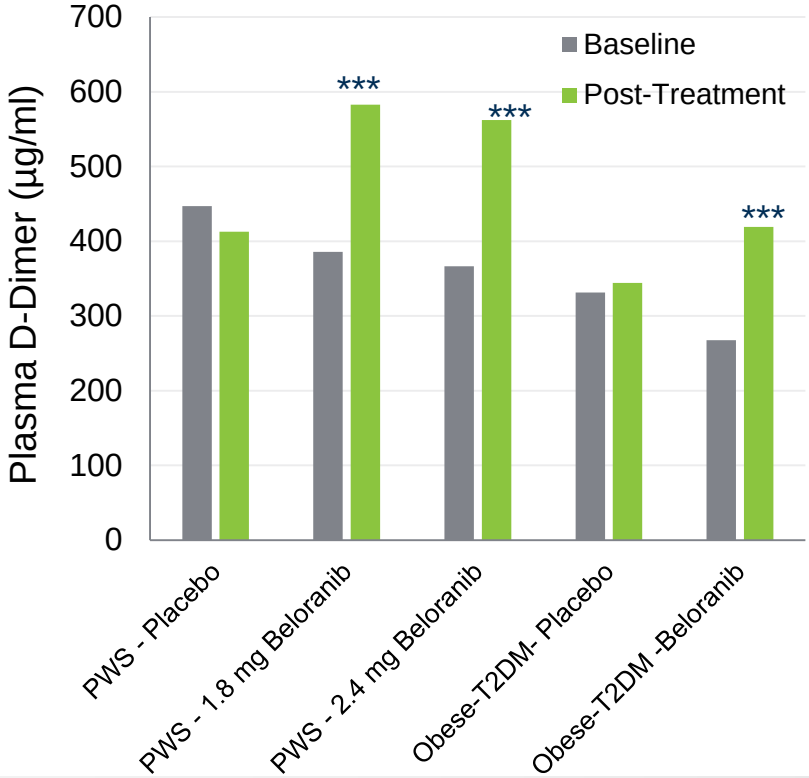


Striking difference in sensitivity to beloranib vs. ZGN-1061 – correlates with differential sensitivity *in vivo*

ZGN-1061 Differentiation: No Impact on D-Dimer Thrombosis Marker

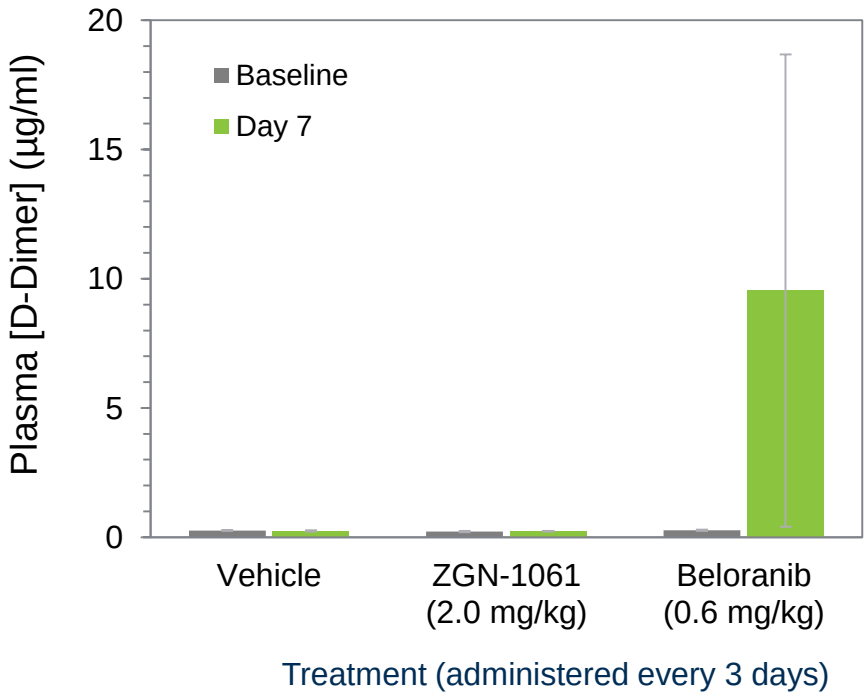
- **D-Dimer levels were increased by beloranib treatment in patients in Phase 2b/3 clinical trials**

Changes observed at earliest time points assessed
(12 weeks in PWS, 15 weeks in Obese-T2DM)



- **Even at high doses, ZGN-1061 is rapidly eliminated and does not elevate D-Dimer or cause clinical signs of thrombosis in dogs**

Plasma levels of thrombosis marker D-dimer following one week of treatment with ZGN-1061 or beloranib



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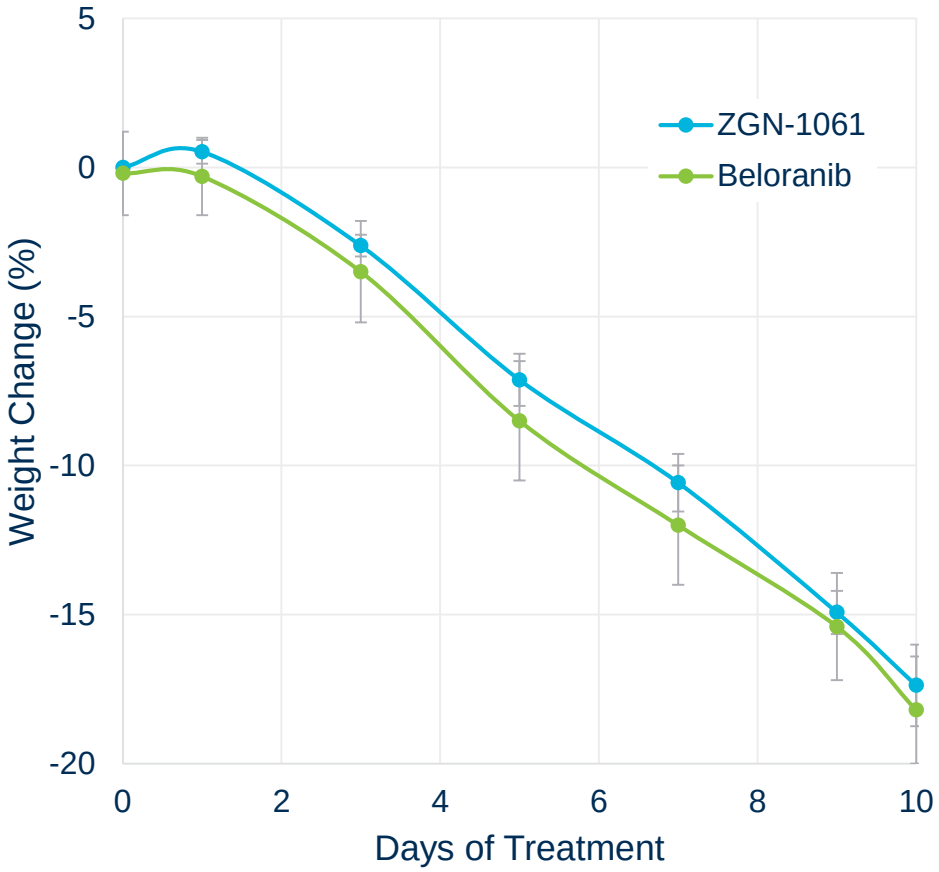
ZGN-1061: Similar Impact on Multiple Metabolic Measures *in vivo* vs. Beloranib



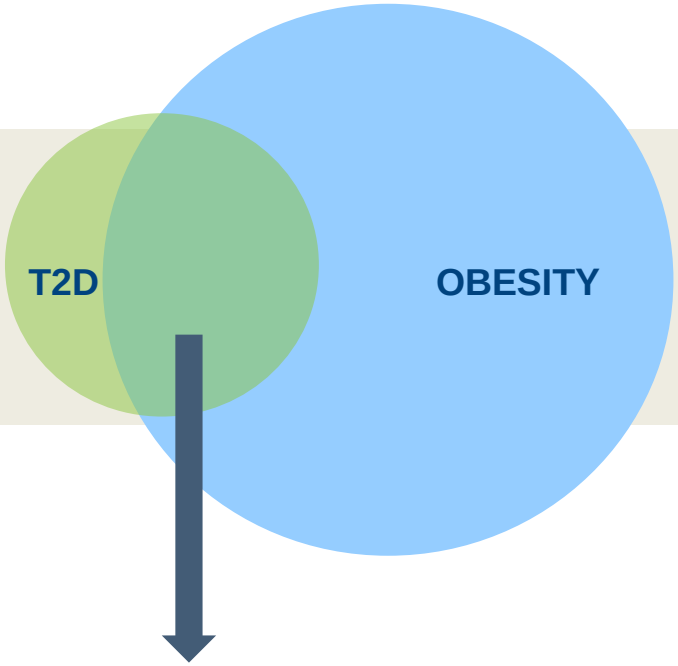
ZGN-1061 and beloranib have similar impact on metabolic parameters in pre-clinical models

- Body weight
- Food intake
- Plasma lipids
- Blood glucose
- Liver function tests

Obese high fat diet-fed C57Bl/6 were treated with ZGN-1061 or beloranib for 10 days by subcutaneous injection at doses leading to similar plasma drug exposures (0.1 mg/kg beloranib vs. 0.3 mg/kg ZGN-1061). Values represent vehicle-adjusted weight change and means ± SEM for n=4 mice per group.

Unmet Medical Need in Type 2 Diabetes and Obesity

Type 2 Diabetes Market
- 25M patients
- $35B Rx market
- 80% of patients receive Rx
- More treatments needed due to progressive nature of disease



Obesity market
- 100M patients
- $500M Rx market
- 5% of patients receive Rx
- Few treatment options

Opportunity for ZGN-1061 to address patient segments of high unmet need

Patients who have failed numerous Rx options/procedures (bariatric surgery failures, patients who require insulin)

Patient segments defined by excess weight, poor glycemic control, inflammation, hyperlipidemia, and fatty liver

ZGN-1061 Clinical Development

Dennis Kim, M.D., Chief Medical Officer

ZGN-1061 Phase 1 Clinical Trial Design

Completed

Part 1: Single Ascending Dose (SAD) Study

- Healthy volunteers
- 6 cohorts (N=~48); N=6 active/2 placebo per cohort
- ~14 day interval between each dose level

Ongoing

Part 2: Multiple Ascending Dose (MAD) Study

- Healthy obese volunteers
- 3 cohorts (N=~24); N=6 active/2 placebo per cohort
- Twice-weekly SC dosing for 28 days (8 injections)

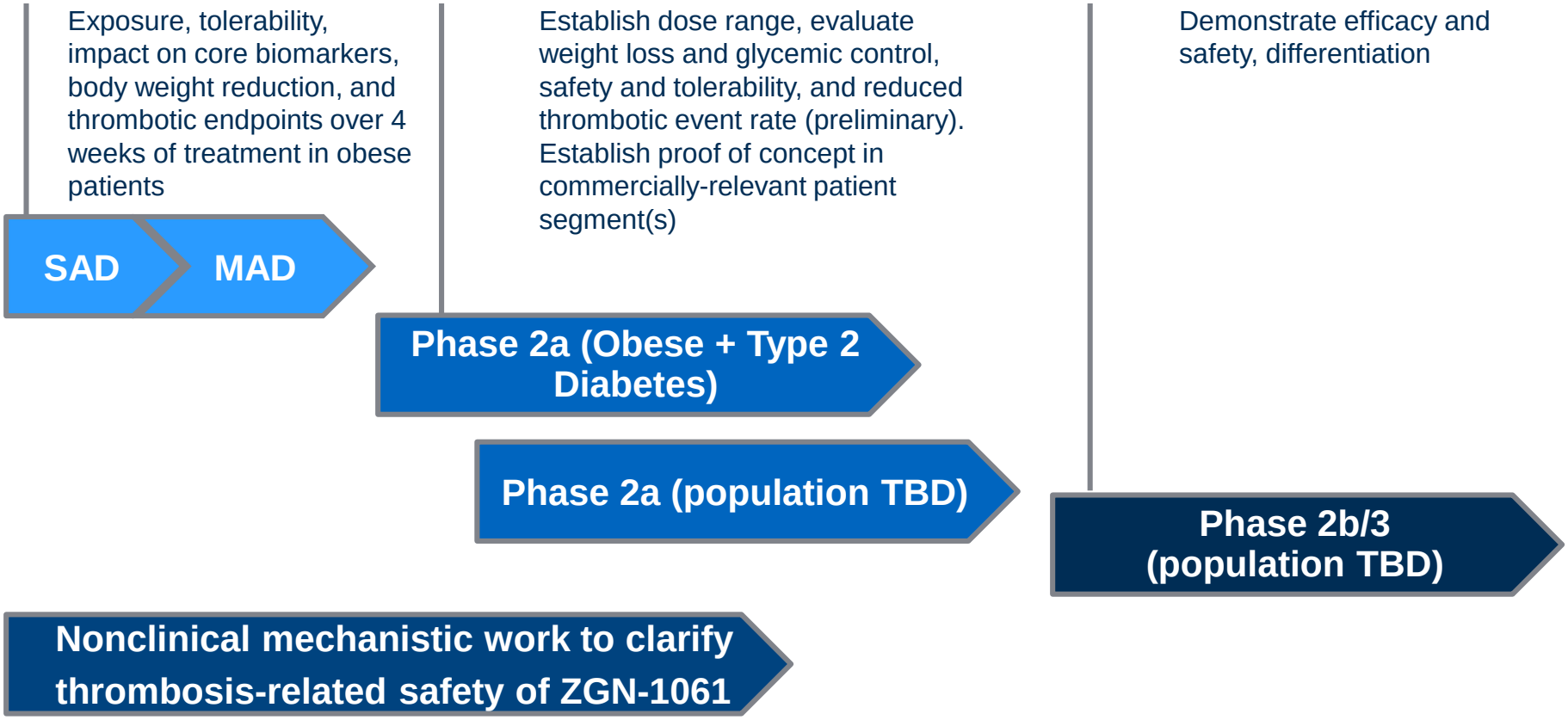
Completion of Dosing of Phase 1 Clinical Trial Expected by End of Q1 2017

Key Outcome Measures for ZGN-1061 Phase 1 Clinical Trial

Beyond standard Phase 1 outcome measures, clinical trial designed to assess endpoints associated with MetAP2i, while advancing understanding of differentiation vs. beloranib

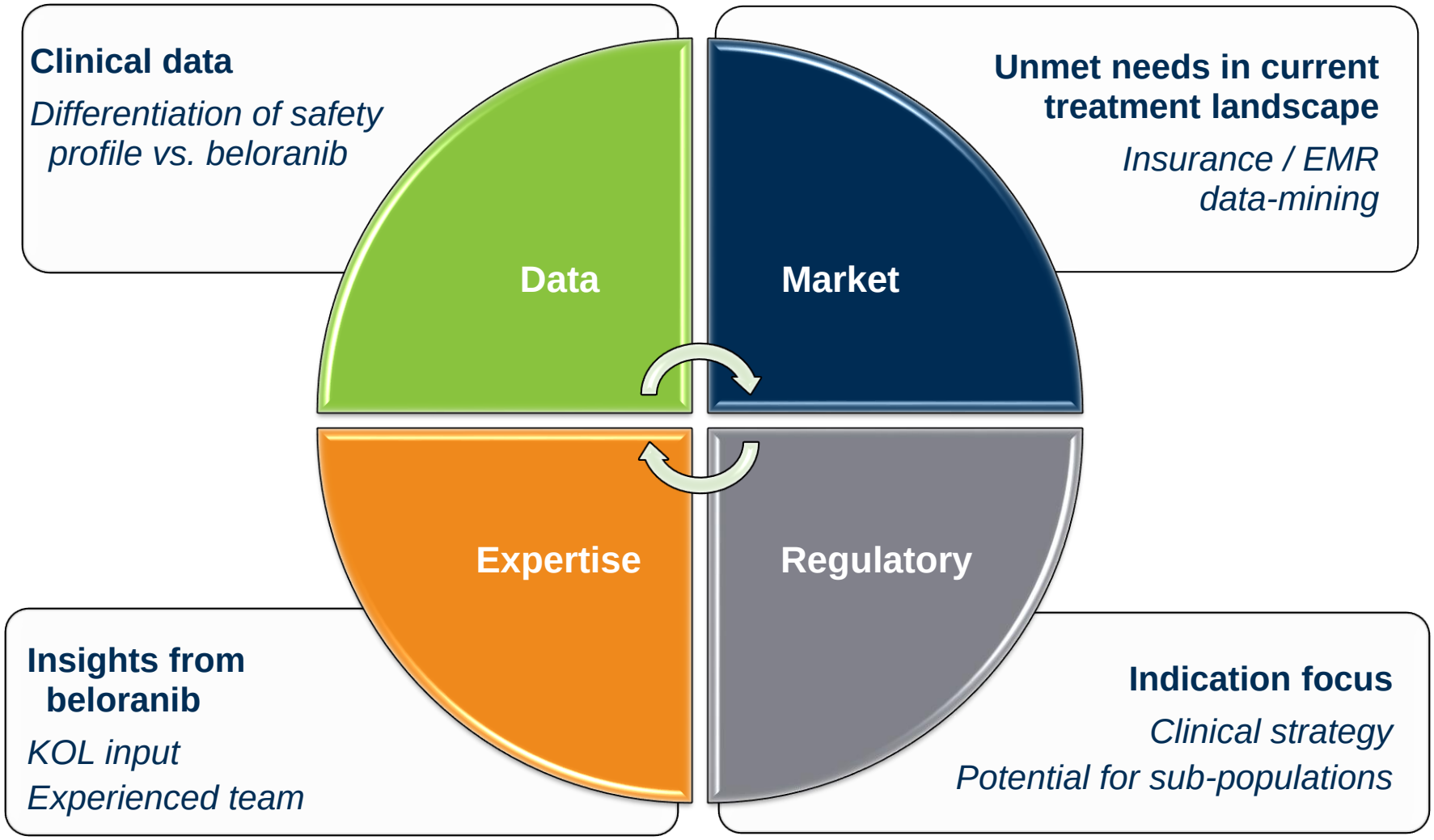
Primary Endpoint	• Safety and tolerability
Pharmacokinetics	• Characterize and confirm improved pharmacokinetic profile for ZGN-1061
Exploratory Efficacy Signals	• Body weight, fat mass, waist and hip circumference, food intake, self-reported appetite, lipids, and other blood markers
Thrombosis Risk	• Drug exposure/PK profile, coagulation biomarkers, thrombotic endpoints

Efficient Core Clinical Program for ZGN-1061*



*Clinical development plan concept to be finalized pending additional review and health authority input

2017 - Informing the Future Development Path



Financials

Patty Allen, Chief Financial Officer

2016 Selected Financial Summary

Balance Sheets	As of Dec. 31, 2016	As of Dec. 31, 2015		
Cash, Cash Equivalents and Marketable Securities	$ 129.2M	$ 185.1M		
Total Assets	$131.6M	$ 189.1M		

Statements of Operations	Quarter Ended Dec. 31, 2016	Quarter Ended Dec. 31, 2015	Year Ended Dec. 31, 2016	Year Ended Dec. 31, 2015
Research & Development Expenses	$ 7.3M	$ 17.7M	$ 39.9M	$ 54.6M
G&A Expenses	$ 3.2M	$ 5.5M	$ 18.3M	$ 19.2M
Net Loss	($ 10.4)M	($ 23.2)M	($ 57.9)M	($ 74.3)M
Net Loss per share	($0.38)	($0.85)	($2.12)	($2.78)

Expect to end 2017 with greater than $65 million in cash, cash equivalents & marketable securities

- Strong position to achieve key value-creating milestones for ZGN-1061

Closing Comments

Tom Hughes, Ph.D., President and Chief Executive Officer

2017 R&D Objectives

MetAP2 Portfolio

- ☐ Ongoing research and discovery focused on MetAP2 pathway

ZGN-1061

- ☐ Complete dosing of ZGN-1061 Phase 1 clinical trial by end of Q1; report data in early Q2
- ☐ Report data package on differentiation of ZGN-1061 from beloranib
- ☐ Initiate Phase 2 clinical trial of ZGN-1061 in obesity and type 2 diabetes in Australia
- ☐ Abstracts and presentations regarding ZGN-1061 clinical and nonclinical profile
- ☐ Refine manufacturing to provide Phase 2 and Phase 3 drug supply

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Thank You

zafgen.com | +1 (617) 622-4003
175 Portland Street, 4th Floor, Boston, MA 02114