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Q4 & Full Year 2015 Financial Results & Business Update

March 9, 2016

Q4 & Full Year 2015 Financial Results

Prepared Remarks

- **Q4 Update**
  - Tom Hughes, Ph.D., Chief Executive Officer
- **Clinical Update**
  - Dennis Kim, M.D., Chief Medical Officer
- **Financial Results**
  - Patty Allen, Chief Financial Officer

Question and Answer Session

- **Also available for Q&A**
  - Patrick Loustau, President
  - Alicia Secor, Chief Commercial Officer

Forward Looking Statements

These slides and the accompanying oral presentation contain forward-looking statements and information. The use of words such as “may,” “might,” “will,” “should,” “expect,” “plan,” “anticipate,” “believe,” “estimate,” “project,” “intend,” “future,” “potential,” or “continue,” and other similar expressions are intended to identify forward looking statements. For example, all statements we make regarding the initiation, timing, progress and results of our preclinical and clinical studies and our research and development programs, our ability to advance product candidates into, and successfully complete, clinical studies, the timing or likelihood of regulatory filings and approvals, and our expected cash, cash equivalents and marketable securities at year end are forward looking. All forward-looking statements are based on estimates and assumptions by our management that, although we believe to be reasonable, are inherently uncertain. All forward-looking statements are subject to risks and uncertainties that may cause actual results to differ materially from those that we expected. These statements are also subject to a number of material risks and uncertainties that are described in our most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission, as updated by our future filings with the Securities and Exchange Commission. Any forward-looking statement speaks only as of the date on which it was made. We undertake no obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future events or otherwise, except as required by law.

Q4 Update

Tom Hughes, Ph.D.
Chief Executive Officer

Recent Clinical Developments

bestPWS Phase 3 ZAF-311 Data	Phase 2b ZAF-203 Data
Prader-Willi Syndrome	Severe Obesity with Type 2 Diabetes
• Achieved both co-primary endpoints • Statistically significant and meaningful reduction in hyperphagia-related behaviors and body weight	• Achieved primary, key secondary endpoints • Statistically significant and clinically meaningful improvements in body weight and glycemic control

Provides additional insight on beloranib's efficacy, safety and tolerability in high-risk patients

Establishing the Benefit / Risk Relationship and Mitigation Plan for Beloranib in PWS

Items to be Submitted to FDA in Effort to Resolve Hold

✓	**bestPWS ZAF-311 Data**
✓	**ZAF-203 Data in Severe and Complicated Obesity**
Developing	**Clinical and Non-clinical Risk Assessment**
Developing	**Risk Mitigation Strategy**

Demonstrate Efficacy: Impact of Treatment, Clinical Relevance and Robustness of Effects

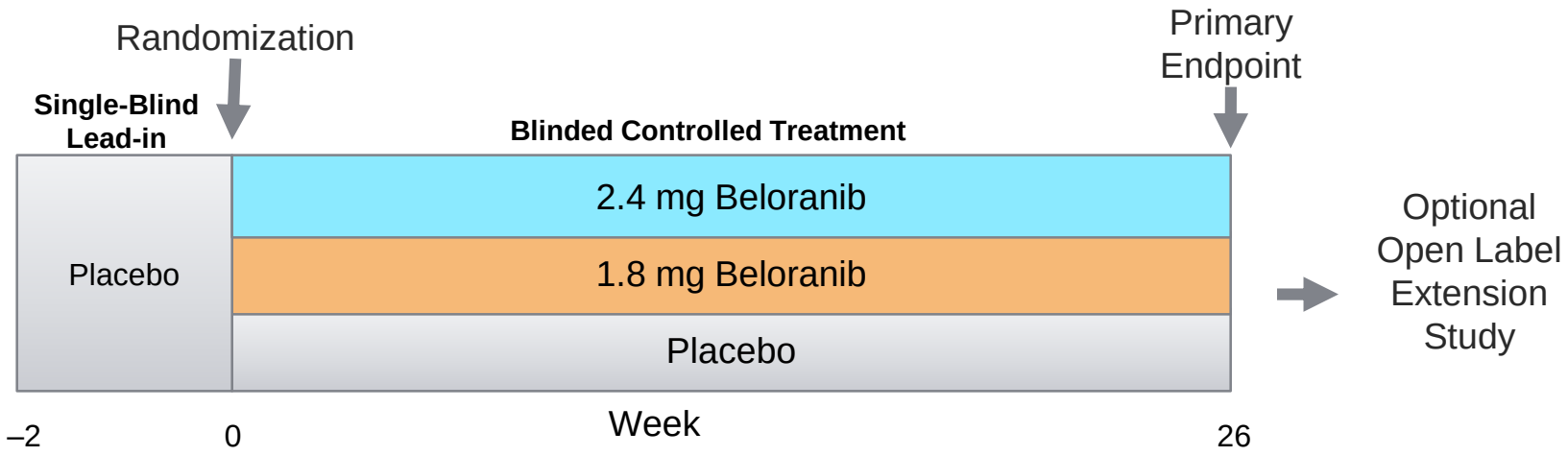
Demonstrate Risk / Benefit from Full Program; Integrated AE Profile

Risk Mitigation Proposal to Screen / Monitor / Mitigate Thrombotic Risk

Clinical Update

Dennis Kim, M.D.
Chief Medical Officer

bestPWS Pivotal Phase 3 ZAF-311 Study Design

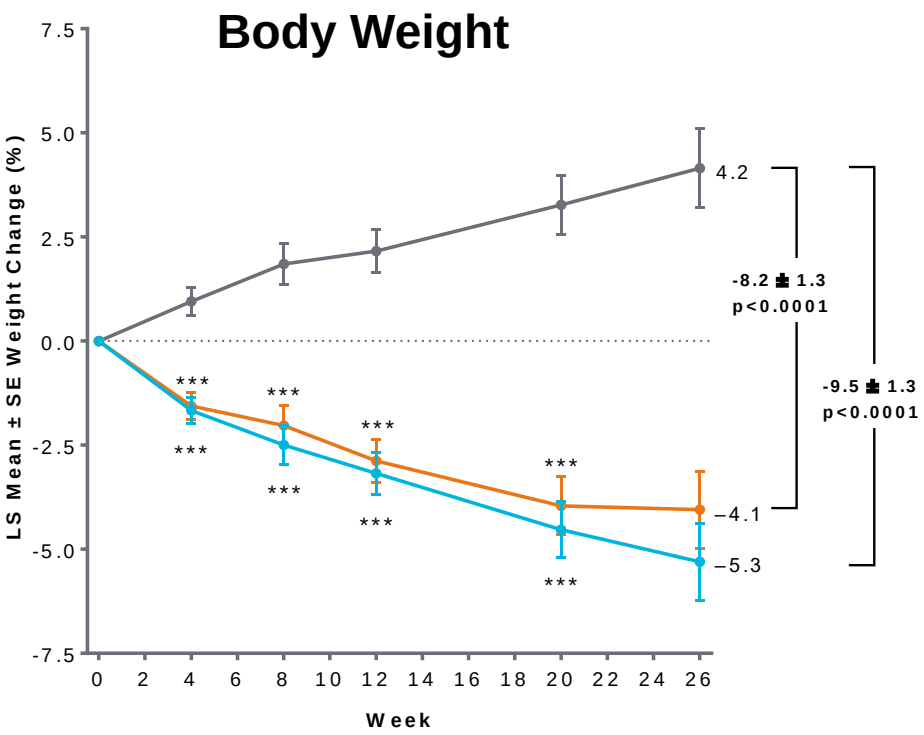


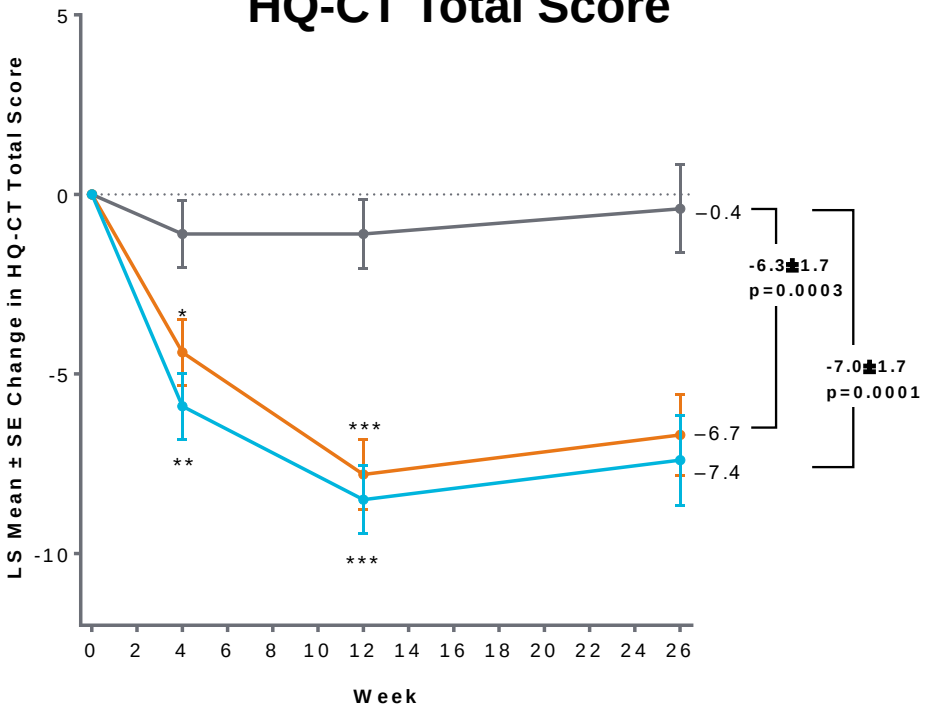
- Co-primary endpoints: improvement in hyperphagia-related behaviors and body weight
- Secondary endpoints: body fat mass, LDL-c, HDL-c, C-reactive protein
- 107 patients randomized
  - Baseline characteristics well-balanced among the three treatment groups
  - Study population representative of general PWS population

Placebo includes placebo low-volume and placebo high-volume. Subjects randomized to 2.4 mg beloranib and all subjects in the OLE received 1.8 mg beloranib for the first 4 weeks of treatment. All doses were administered twice-weekly by subcutaneous injection.

bestPWS Study Achieves Co-Primary Efficacy Endpoints ITT Population (N=107)

- Statistically significant reduction in body weight and hyperphagia at both doses





Placebo (N=34); BL Wt=100.9 kg; BL HQ-CT=15.0

1.8 mg Beloranib (N=36); BL Wt = 97.5 kg; BL HQ-CT=17.4

2.4 mg Beloranib (N=37); BL Wt=105.7 kg; BL HQ-CT=18.3

*p<0.05, **p<0.01, ***p<0.0001 for change from baseline with beloranib vs. placebo.
Analysis is implemented via a mixed model repeated measures (MMRM) model.

bestPWS Frequent Treatment-Emergent Adverse Events (TEAE) and Serious Adverse Events– Safety Population (N=107)

	Placebo N=34	1.8 mg Beloranib N=36	2.4 mg Beloranib N=37
Any TEAE	22 (64.7%)	30 (83.3%)	29 (78.4%)
Injection site bruising*	1 (2.9%)	4 (11.1%)	6 (16.2%)
Aggression*	5 (14.7%)	5 (13.9%)	4 (10.8%)
Hyperphagia*	3 (8.8%)	6 (16.7%)	2 (5.4%)
Any Serious TEAE	2 (5.9%)	2 (5.6%)	1 (2.7%)
Ankle fracture	1 (2.9%)	0	0
Aggression	1 (2.9%)	0	1 (2.7%)
Mental status changes	0	1 (2.8%)	0
Pulmonary embolism	0	1 (2.8%)	0
Any TEAE leading to withdrawal of study drug prior to 10/16/15	0	4 (11.1%)	2 (5.4%)
Abnormal behavior	0	1 (2.8%)	0
Anxiety	0	1 (2.8%)	0
Injection site pain	0	0	1 (2.7%)
Mental status changes	0	1 (2.8%)	0
Psychotic disorder	0	0	1 (2.7%)
Pulmonary embolism	0	1 (2.8%)	0
TEAE Leading to Death	0	1 (2.8%)	0

Data are number (%) for the Safety Population, which includes all subjects who received at least 1 dose of randomized study drug. * Treatment-emergent adverse events with incidence ≥10% in combined beloranib group.

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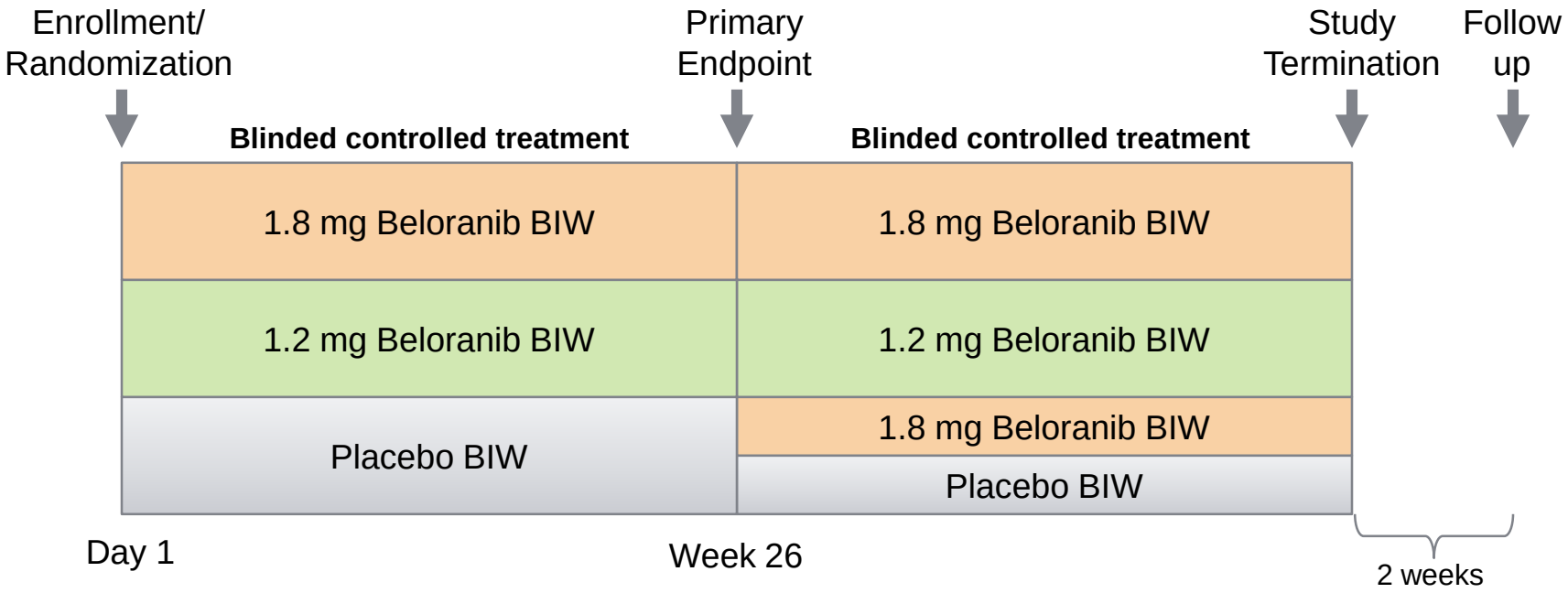
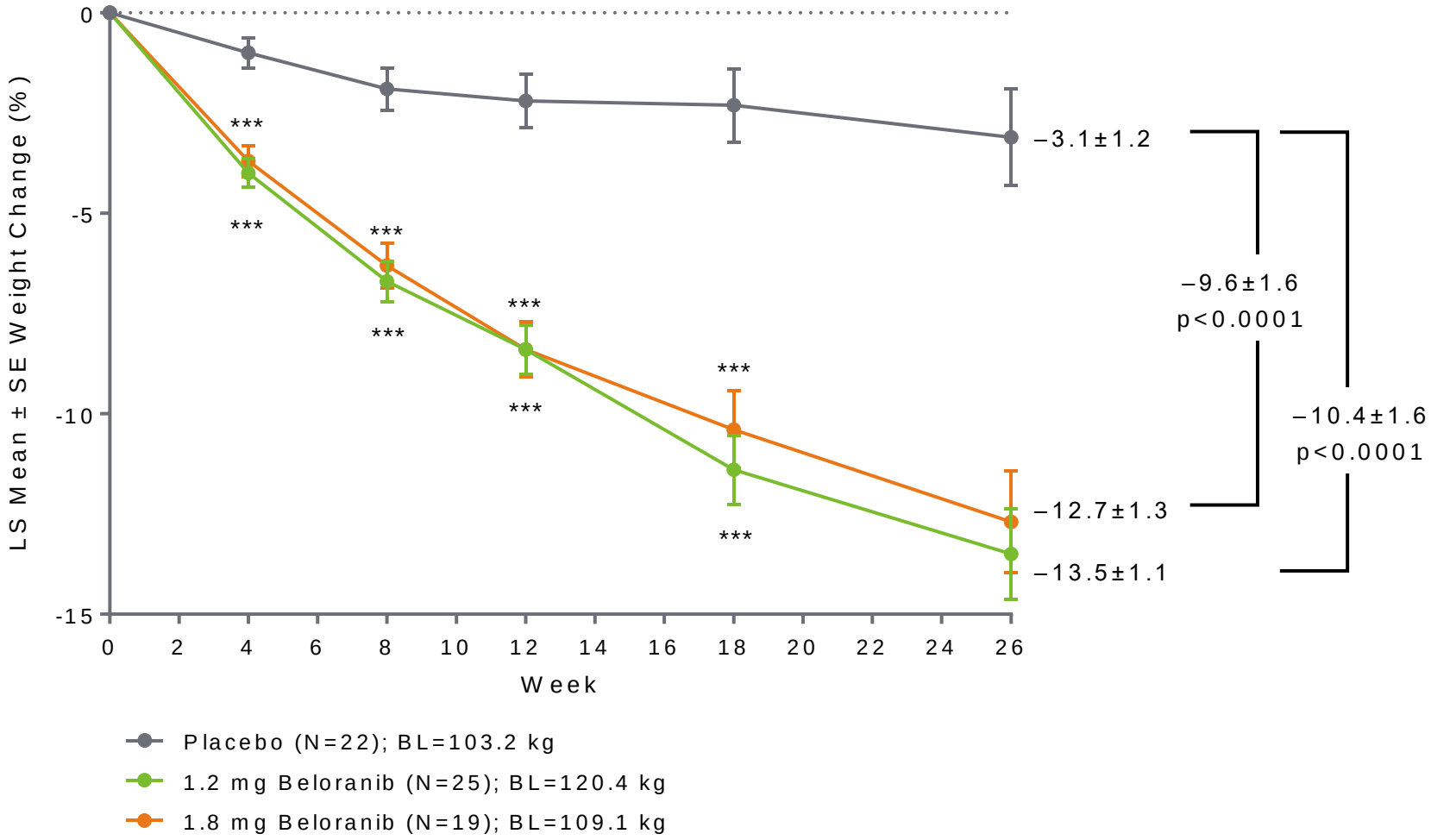
ZAF-203: Clinical Study Design



- Primary efficacy endpoint: Absolute and percent weight at Week 26
- Secondary endpoints included change in fasting glycemic parameters
- 152 patients randomized; 66 patients completed 6 months of treatment prior to suspension of study

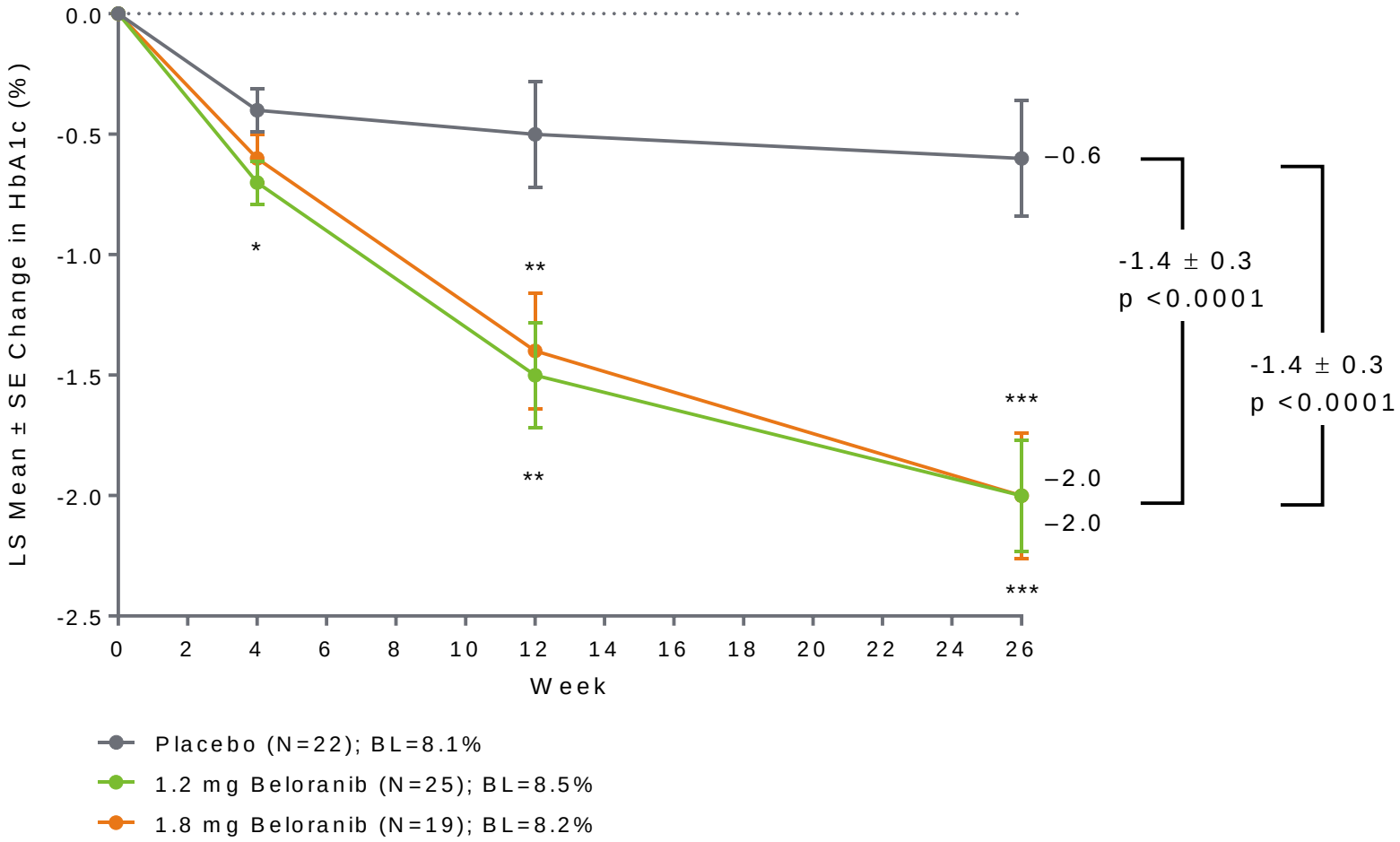
Placebo includes placebo low-volume and placebo high-volume. Subjects randomized to 1.8 mg Beloranib received 1.2 mg Beloranib for the first 4 weeks of each blinded treatment period. Follow up assessments were conducted at 4 days and 2 weeks after study termination. Study interruption by clinical hold focused week 26 evaluation on per protocol population of 66 patients.

ZAF-203: Beloranib Resulted in Statistically Significant Weight Loss vs. Placebo (PP Population)



*p<0.05, **p<0.01, ***p<0.0001 for change from baseline with beloranib vs. placebo.

ZAF-203: Beloranib Treatment Improved Glycemic Control Over 26 Weeks (PP Population)



*p<0.05, **p<0.01, ***p<0.0001 for change from baseline with beloranib vs. placebo.

ZAF-203 Frequent Treatment Emergent Adverse Events Safety Population (N=152)

Adverse Event	Placebo N=51	1.2 mg Beloranib N=52	1.8 mg Beloranib N=49	Placebo/1.8 mg Beloranib N=10
Any TEAE	43 (84.3%) 235	48 (92.3%) 268	41 (83.7%) 229	9 (90.0%) 21
Upper respiratory tract infection	10 (19.6%) 12	15 (28.8%) 19	7 (14.3%) 8	1 (10.0%) 1
Diarrhoea	8 (15.7%) 8	10 (19.2%) 12	9 (18.4%) 12	2 (20.0%)2
Injection site bruising	7 (13.7%) 11	10 (19.2%) 10	7 (14.3%) 10	0
Abnormal dreams	1 (2.0%) 1	8 (15.4%) 8	4 (8.2%) 4	1 (10.0%)
Sleep disorder	1 (2.0%) 1	5 (9.6%) 5	7 (14.3%) 7	0
Lower respiratory tract infection	2 (3.9%) 2	7 (13.5%) 7	3 (6.1%) 3	0
Nausea	11 (21.6%) 13	5 (9.6%) 6	4 (8.2%) 5	1 (10.0%) 1
Headache	9 (17.6%) 14	7 (13.5%) 9	2 (4.1%) 2	0
Cough	2 (3.9%) 2	3 (5.8%) 3	5 (10.2%) 5	0
Injection site erythema	1 (2.0%) 3	2 (3.8%) 2	6 (12.2%) 7	0

Data are number of patients, (%), and number of events for the Safety Population, which includes all subjects who received at least 1 dose of randomized study drug. Placebo/1.8 mg beloranib includes any events that began after Week 26 (on 1.8 mg beloranib) and are the same subjects represented within the placebo column. Terms were included if ≥10% incidence in the placebo, 1.2 mg beloranib or 1.8 mg beloranib treatment arms.

Overview of Adverse Events (AE) and Serious Adverse Events (SAE) Related to Thrombosis

Beloranib IND was placed on a full clinical hold by the FDA on December 2, 2015. Six AE and five SAE related to thrombosis observed across nine clinical trials evaluating >500 patients. Thrombotic events to date seen only in patients randomized to beloranib.

Study	Dose	Event	Causality per Investigator	Additional Info
ZAF-201 (completed)	1.2mg	SAE of pulmonary embolism (PE); thrombophlebitis	Not related	Factor V Leiden mutation
	2.4mg	SAE of PE; Deep vein thrombosis (DVT)	Not related	Gout attack and extended immobilization
	2.4mg	Moderate AE of thrombophlebitis superficial	Not related	Varicose veins; Implanted contraceptive
	2.4mg	Mild AE of thrombophlebitis superficial	Not related	Implanted contraceptive
ZAF-203 (completed)	1.2mg	SAE of PE	Not related	Implanted contraceptive; heart failure; systemic pulmonary inflammatory disease
	1.8mg	Moderate AE of DVT	Related	Discovered during VTE screening, 4 weeks after last dose of study drug. Two 8-hour flights occurring 3-4 weeks prior to VTE screening.
	1.2mg	Moderate AE of thrombophlebitis superficial	Related	Discovered during VTE screening, 19 weeks after last dose of study drug. Ongoing medical history of bilateral superficial venous insufficiency
ZAF-311 (completed)	1.8mg	Moderate AE of thrombophlebitis superficial; DVT	Possibly related	Extended (6 hour) car ride
	2.4mg	Moderate AE of DVT	Possibly related	Androgel 1% transdermal patch
	1.8mg	SAE of PE; death	Possibly related	BMI 55 with multiple co-morbidities
	2.4mg	SAE of PE; death	Probably related	Ongoing thrombophlebitis superficial (prior history); treated with ASA

2015 Financial Results

Patty Allen
Chief Financial Officer

2015 Selected Financial Summary

Balance Sheets	As of December 31, 2015	As of December 31, 2014
Cash, Cash Equivalents and Marketable Securities	$ 185.1M	$ 115.5M*
Total Assets	$ 189.1M	$ 117.5M

Statements of Operations	Year Ended December 31, 2015	Year Ended December 31, 2014
Research & Development Expenses	$ 54.6M	$ 27.4M
General & Administrative Expenses	$ 19.2M	$ 8.1M
Net Loss	($ 74.3)M	($ 36.5)M
Net Loss per Share	($2.78)	($3.00)

- Expect to end 2016 with greater than $100 million in cash
  - Strong position to drive our programs forward
  - Plan to provide more specific guidance following potential resolution of full clinical hold

*Pro-forma cash position at December 31, 2014 of $245 million includes $130 million of net proceeds from follow-on offering in January 2015

Closing Remarks

Tom Hughes, Ph.D.
Chief Executive Officer

Q&A

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Thank You

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