



Second Quarter 2016 Financial Results/Corporate Update

August 4th, 2016

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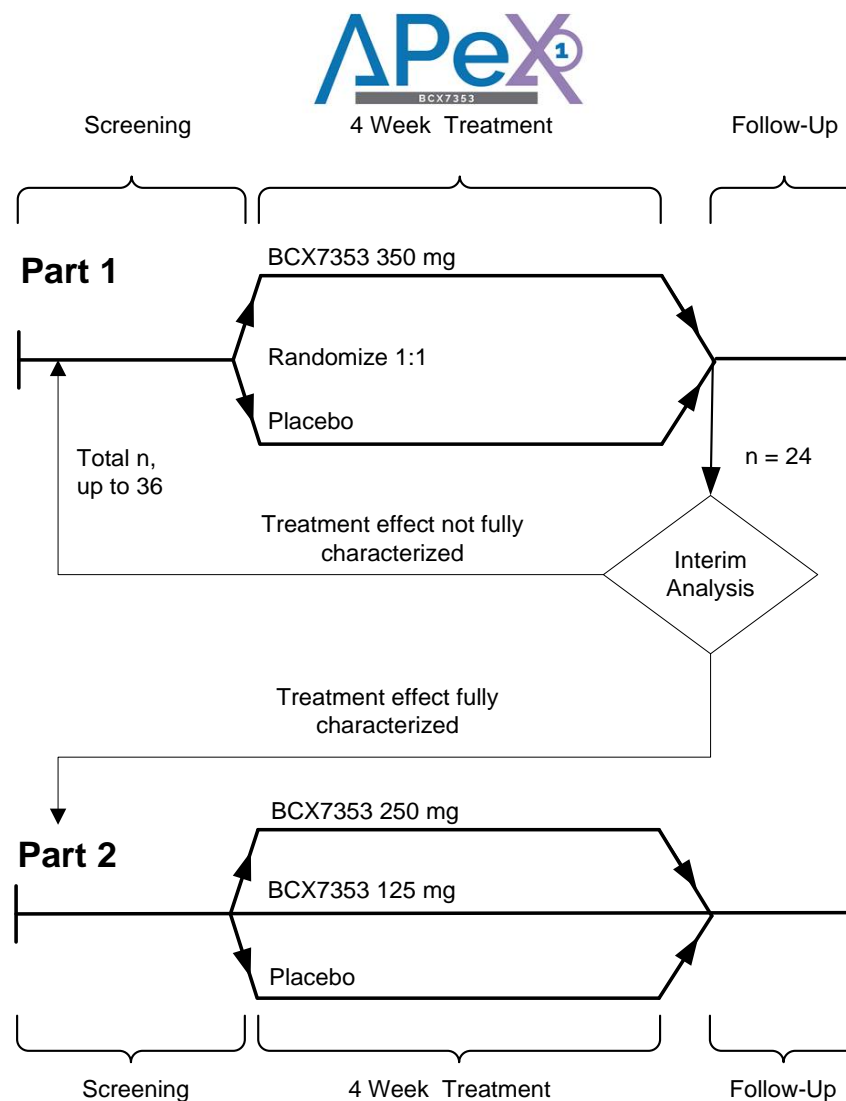
APeX-1: Phase 2 placebo-controlled trial of BCX7353 in HAE patients

Design

- Part 1: proof of concept
 - 350 mg QD BCX7353 vs placebo
 - Interim analysis at n = 24
 - Option to add up to 12 subjects for total n = 36
 - Powered at 90% ($\alpha=0.05$) to detect a reduction in number of HAE attacks of $\geq 70\%$ on BCX7353
- Part 2: dose ranging
 - 250 mg QD and 125 mg QD BCX7353 and placebo
 - n = 14
 - 6:6:2 randomization

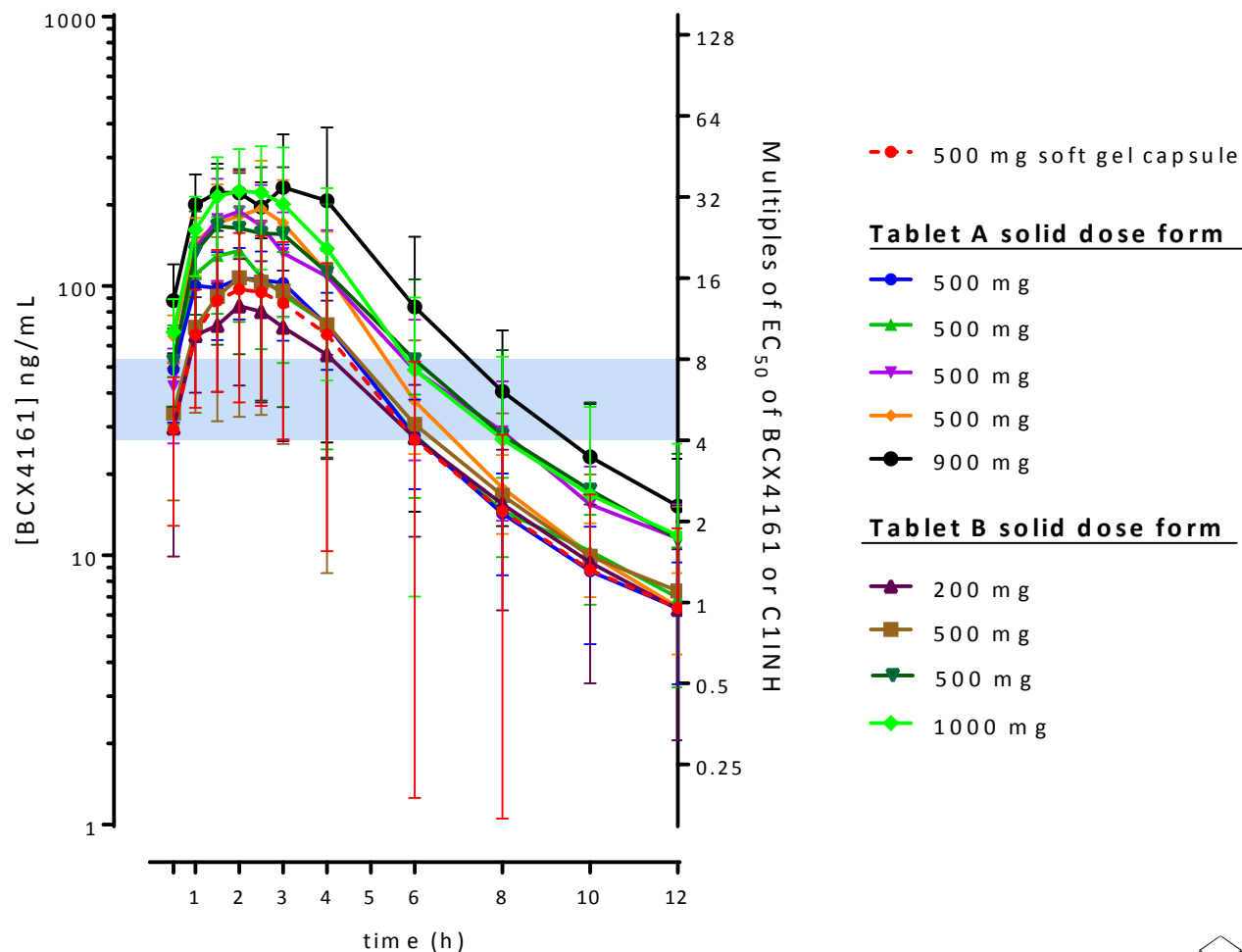
Endpoints

- Number of HAE attacks by treatment group will be analyzed as weekly attack rate, number of attacks, proportion of subjects with no attacks, number of attack-free days
- Additional endpoints include full safety assessments, QOL, PK/PD



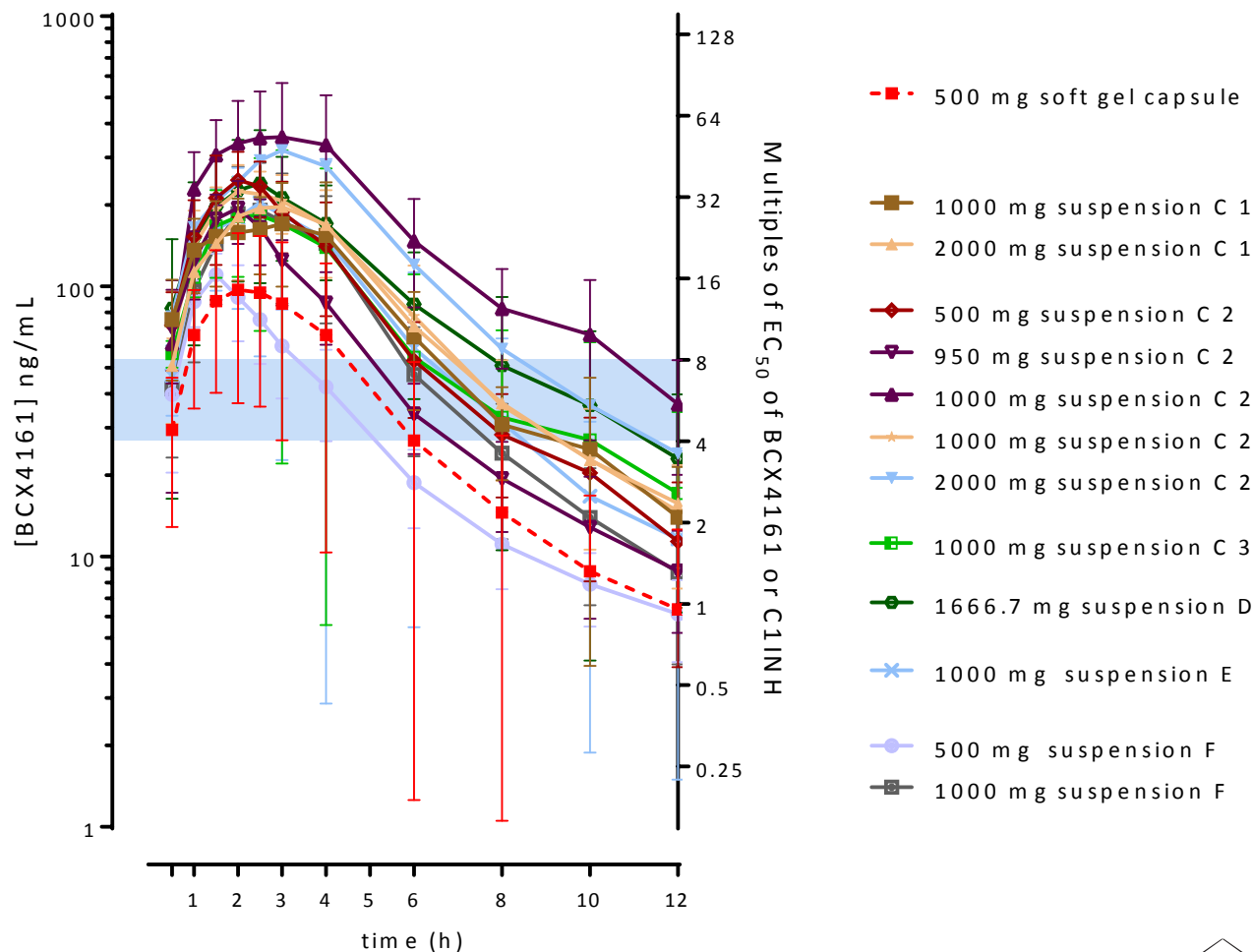
Avoralstat PK profiles after dosing tablet formulations, compared to soft gel capsule formulation

Plasma concentration time profile after dosing tablet formulations
ng/mL, mean (SD)

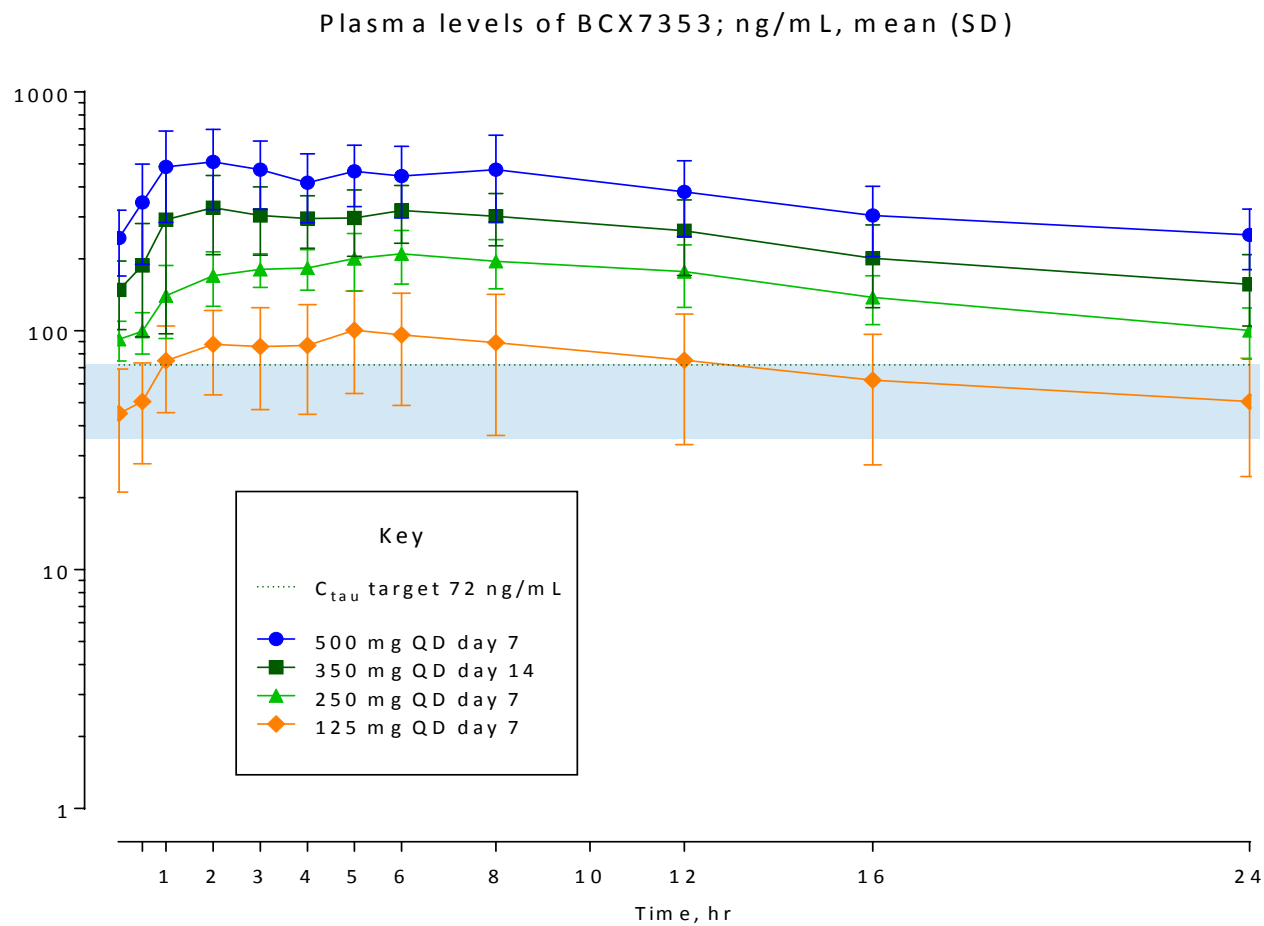


Avoralstat PK profiles after dosing suspension formulations, compared to soft gel capsule formulation

Plasma concentration time profile after dosing suspension formulations
ng/mL, mean (SD)



PK profile of BCX7353 dosed once daily in healthy subjects



First-in-human phase 1 clinical study of broad-spectrum antiviral nucleoside analog BCX4430, administered by intramuscular (i.m.) injection

SAD Cohort	Dose, mg/kg	Number of Subjects
1	0.3	6 active, 2 placebo
2	0.75	6 active, 2 placebo
3	1.8	6 active, 2 placebo
4	4	6 active, 2 placebo
5	7	6 active, 2 placebo
6	10	6 active, 2 placebo
Lidocaine evaluation	4	14 active

MAD Cohort	Dose, mg/kg QD for 7 days	Number of Subjects
1	2.5	7 active, 2 placebo
2	5	8 active, 2 placebo
3	10	8 active, 2 placebo

- Study BCX4430-101 evaluated the safety, tolerability, and pharmacokinetics of 1 dose and 7 days of daily dosing by i.m. injection in 91 healthy volunteers
- All planned cohorts were completed
- Effect of adding lidocaine (local anesthetic) to i.m. injections was also evaluated

Clinical study (ClinicalTrials.gov identifier: NCT02319772) funded with Federal funds under NIH contract HHSN272201300017C

BCX4430 administered by i.m. injection was generally safe and well tolerated over the range of doses and durations tested

Single doses of 0.3 mg/kg through 10 mg/kg

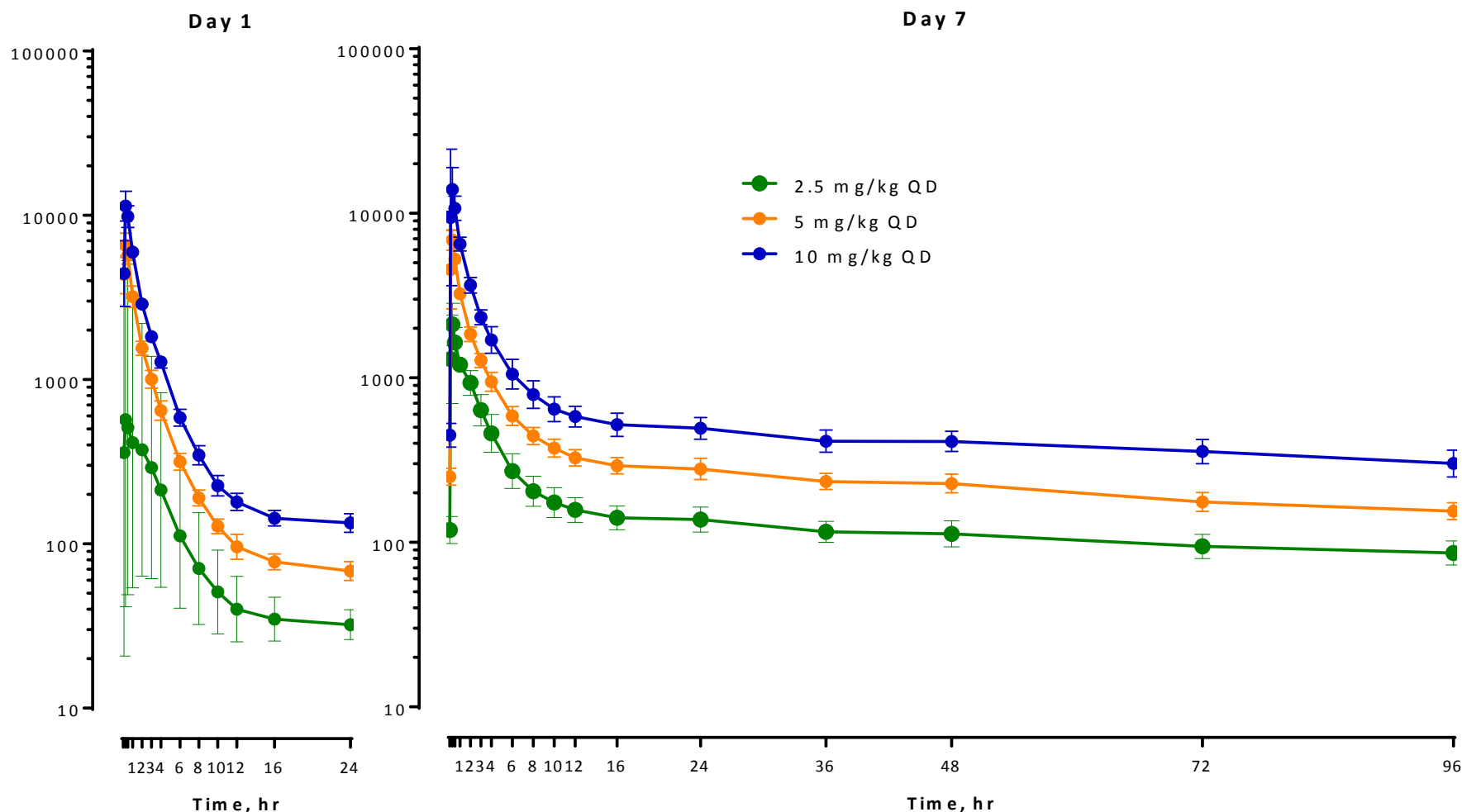
- 50 subjects received single doses of BCX4430 (12 subjects received placebo)
- No serious or severe adverse events occurred
- The most frequently reported AE across all cohorts was injection site pain: 23 subjects (46%)
- No clinically significant laboratory abnormalities occurred at any dose
- Co-administration of lidocaine with BCX4430 was found to ameliorate injection site pain, without altering the plasma PK profile of BCX4430

Once daily doses of 2.5 mg/kg through 10 mg/kg for 7 days

- 23 subjects received daily doses of BCX4430 with lidocaine (6 subjects received placebo)
- All subjects except 1 completed planned dosing through 7 days (one subject developed gastroenteritis unrelated to study drug)
- No serious or severe adverse events occurred
- The most frequently reported AE across all cohorts was injection site pain: 5 subjects (22%)
- No clinically significant laboratory abnormalities occurred at any dose
- With co-administration of lidocaine, the injections were well tolerated

Plasma concentration-time profile of BCX4430 on the first and last day of dosing by daily intramuscular injection

Plasma PK profile of BCX4430 during once-daily dosing by IM injection
ng/mL; geometric mean (95% CI)



Phase 1 study of BCX4430 administered via IM injection in healthy volunteers: conclusions

- The study achieved all of its objectives
- BCX4430 was generally safe and well tolerated at doses up to 10 mg/kg once daily for 7 days
- Exposure was dose-proportional
- These results support the continued development of BCX4430 as a parenterally administered broad-spectrum antiviral drug for the treatment of serious emerging viral infections

Second quarter operating results

	Q2 2016	Q2 2015	Change Q2 2016 vs Q2 2015
<i>(in thousands, except per share amounts)</i>			
Revenues:			
Royalty revenue	\$ 629	\$ 132	377%
Collaborative and other R&D	4,158	25,710	(84%)
Total revenues	4,787	25,842	(81%)
Expenses:			
Research and development	14,166	16,524	(14%)
General and administrative	2,724	3,534	(23%)
Royalty	27	442	(94%)
Total expenses	16,917	20,500	(17%)
(Loss) income from operations	(12,130)	5,342	(327%)
Interest and other income, net	147	116	27%
Interest expense	(1,421)	(1,306)	9%
(Loss) gain on foreign currency hedge	(2,877)	749	(484%)
Net (loss) gain	\$ (16,281)	\$ 4,901	(432%)
Net (loss) gain per share - Basic	\$ (0.22)	\$ 0.07	(414%)
Net (loss) gain per share - Diluted	\$ (0.22)	\$ 0.06	(467%)
Net operating cash utilization	\$ 15,446	\$ 11,953	29%
Weighted avg shares outstanding - basic	73,695	72,642	
Weighted avg shares outstanding - diluted	73,695	76,760	

Six month operating results

	1H 2016	1H 2015	Change 2016 vs 2015
<i>(in thousands, except per share amounts)</i>			
Revenues:			
Product sales, net	\$ —	\$ 537	(100%)
Royalty Revenue	2,519	1,650	53%
Collaborative and other R&D	7,088	30,481	(77%)
Total revenues	9,607	32,668	(71%)
Expenses:			
Cost of products sold	—	15	(100%)
Research and development	34,745	33,644	3%
General and administrative	5,936	7,595	(22%)
Royalty	104	502	(79%)
Total expenses	40,785	41,756	(2%)
Loss from operations	(31,178)	(9,088)	243%
Interest and other income, net	586	233	152%
Interest expense	(2,891)	(2,621)	10%
(Loss) gain on foreign currency hedge	(5,630)	1,213	(564%)
Net loss	\$ (39,113)	\$ (10,263)	281%
Net loss per share - Basic & Diluted	\$ (0.53)	\$ (0.14)	279%
Net operating cash utilization	\$ 37,891	\$ 15,802	140%
Weighted average shares outstanding	73,648	72,492	

Cash position & 2016 guidance (in millions)

Cash & investments at December 31, 2015	\$100.9
Operating cash utilization through June 30, 2016	(\$37.9)
Cash & investments at June 30, 2016	\$64.3

2016 Guidance

Operating cash utilization	\$55 – 75
Operating expenses [#]	\$78 – 98
Cash runway	Mid-2017

[#] Excludes equity-based compensation.