ROTH Capital Partners

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Healthcare

California Dreamin' II: Good Things In Small Packages

As we approach our Annual ROTH Conference at the Ritz-Carlton in sunny Dana Point, California on March 17-19, below we highlight some of our smaller participating companies under coverage and some key events and themes around them. Reply to this email or call us to schedule 1x1 meetings in California with any and/or all of them.

Oryzon Genomics (MC €143M). *Emerging therapeutic class*: epigenetics. *Upcoming*: First data from the Phase 2a basket study of vafidemstat in aggression in 2Q; first data from the Phase 2a of iadademstat in AML and SCLC in 2Q-3Q; preliminary data from the Phase 2a of vafidemstat in Alzheimer's and MS in 2H. *Highlight*: The bifurcated application of LSD1 inhibitors in oncology and neuro rests on an increasing body of supporting science, and offers opportunities for both orphan indications and broad markets in each of these two fields independently.

Anavex Life Sciences (MC $141M). *Hot therapeutic area*: neurodegeneration. *Upcoming*: POC data from the Phase 2 of Anavex 2-73 in the orphan Rett Syndrome around end-2019; data from the Phase 2 in Parkinson's dementia in early 2020; data from the Phase 2b/3 in Alzheimer's around end-2020. *Highlight*: The agent engages a novel target (sigma-1) and is hedged in both orphan neurodevelopmental disorders and biomarker-selected neurodegenerative disease, improving qualitatively and quantitatively the odds of success in an otherwise challenging field.

Actinium Pharmaceuticals (MC $68M). *Hot therapeutic area*: bone marrow transplant conditioning & lymphodepletion. *Upcoming*: DMC safety analysis of pivotal SIERRA study in 1H19 (with readthrough on efficacy), and interim efficacy in 2H19; advance of anti-CD33 agent in transplant conditioning in MDS; advance of anti-CD45 agent in lymphodepletion for cell therapies. *Highlight*: Bone marrow transplant conditioning is an underappreciated and underpopulated therapeutic niche, which may drive meaningful value for Actinium as early as 2H19 after the SIERRA interim look (we are bullish on this study based on the clinical updates at ASH and TCT). Meanwhile, the new program around lymphodepletion could generate validating partnerships given its broad potential applicability across the CAR-T space.

Aptose Biosciences (MC $61M). *Emerging therapeutic class*: reversible BTK inhibitor. *Upcoming*: Data from the dose escalation of the MYC inhibitor APTO-253 in AML; start of dose escalation study of the dual FLT3 and BTK inhibitor CG'806, headed in parallel towards AML and CLL. *Highlight*: The MYC inhibitor tackles a key oncology target, and could pick up and succeed where bromodomain inhibitors failed and left off (due to tox). Importantly, it could be a monetizable asset upon successful target engagement and clinical POC data. Meanwhile, the dual kinase inhibitor has potential in both myeloid and lymphoid disease, with populations resistant to FLT3 and BTK inhibitors representing a quick and efficient development and regulatory path forward.

Moleculin Biotech (MC $34M). *Emerging therapeutic class*: STAT3 inhibitor. *Upcoming*: Initial data from the Phase 1/2 study of the liposomal anthracycline Annamycin in AML; initial clinical data from the first-in-class STAT3 inhibitor WP1066 in glioblastoma, and IND of the next-gen STAT3 inhibitor WP1732. *Highlight*: This small company has two assets in the clinic already, and is working on the IND of a third one. Based on its mode of action and parallels to other known therapies, we expect the liposomal anthracycline to generate positive data in R/R AML (potentially a RP2D in 2019). Meanwhile, the STAT3 portfolio offers both development and licensing optionality.

Cyclacel Pharmaceuticals (MC $11M). *Emerging therapeutic class*: CDK2/9 & MCL1. *Upcoming*: Clinical update from the expansion cohort of the Phase I of sapacitabine and seliciclib in metastatic breast cancer with BRCA1/2 mutations; first data from the Phase 1 study of the CDK2/9 inhibitor CYC065 in combination with venetoclax in AML. *Highlight*: We guide attention to CYC065 (currently being evaluated at MD Anderson), and its novel direct target CDK2/9 and its indirect molecular target MCL-1, which should give it biologic and clinical synergy with BCL-2 inhibitors (such as venetoclax) in CLL, as well as optionality in other tumors associated with MCL-1, MYC, and Cyclin-E.

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For important disclosure information regarding the companies in this summary report, please contact: The Director of Research at (800) 678-9147 or write to: ROTH Capital Partners, LLC, Attention: Director of Research, 888 San Clemente Drive, Newport Beach, CA 92660

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Distribution of IB Services Firmwide

			IB Serv./Past 12 Mos. as of 03/07/19	
Rating	**Count**	**Percent**	**Count**	**Percent**
Buy [B]	**266**	**76.88**	**142**	**53.38**
Neutral [N]	**51**	**14.74**	**31**	**60.78**
Sell [S]	**3**	**0.87**	**1**	**33.33**
Under Review [UR]	**26**	**7.51**	**12**	**46.15**

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