

28 Oct 2016 |

PIPELINE WATCH: Phase III Trials Start In Atypical HUS, Fabry Disease And Colorectal Cancer

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Pipeline Watch has been updated to bring greater depth and detail to a potential therapeutic product's progress through the R&D pipeline. The table brings you a snapshot of selected late-stage clinical trial events in the pharma and biotech industries.

The table records clinical developments using data from Informa Pharma Intelligence's Biomedtracker. It lists drugs for which an important event was recorded between October 21 and October 27, 2016, divided by event type.

Events can include Phase II and Phase III trial initiations, development discontinuations, and the release of top-line and full clinical trial results. A brief summary of product approvals is also included.

A companion resource, The Pink Sheet's regulatory-focused FDA Performance Tracker, follows regulatory milestones such as new submissions and user fee calculations, complete response letters and breakthrough therapy designations.

PIPELINE WATCH – October 21 to October 27, 2016

Lead company/partner	Compound	Indication	Comments
Phase III Suspended			
<i>Dipexium Pharmaceuticals Inc.</i>	<i>Locilex</i> (pexiganan) cream	diabetic foot ulcer infections	OneStep-1 and -2; did not meet primary endpoint.
<i>Inovio Pharmaceuticals</i>	VGX-3100	cervical dysplasia	FDA clinical hold on

<u>Inc.</u>			proposed trial due to concerns about delivery device.
<u>AstraZeneca PLC</u>	durvalumab	head and neck cancer	<u>FDA partial clinical hold due to bleeding events.</u> <u>New patient enrolment halted.</u>
Phase III Results			
<u>Janssen Pharmaceutical Cos. /Bayer AG</u>	Xarelto (rivaroxaban)	treating and preventing venous thromboembolism	Real world data confirms safety and efficacy.
<u>Sunovion Pharmaceuticals Inc.</u>	Latuda (lurasidone)	schizophrenia in adolescents	Safe and effective, supplemental NDA submitted.
<u>Vertex Pharmaceuticals Inc.</u>	Okambi (lumacaftor, ivacaftor) and Kalydeco (ivacaftor)	cystic fibrosis	They modify progression in long-term data.
<u>Chimerix Inc.</u>	brincidofovir	adenovirus infection	AdVise; viremia declined in blood cell transplant patients.
<u>Gilead Sciences Inc.</u>	Descovy (emtricitabine and tenofovir alafenamide)	HIVAIDS	Switching from Truvada (emtricitabine and tenofovir disoproxil fumarate)
Phase III Interim/Top-line Results			
<u>Merck & Co. Inc.</u>	Keytruda (pembrolizumab)	advanced bladder cancer	KeyNote-045; improved overall survival.
AstraZeneca PLC	Lynparza (olaparib)	ovarian cancer	<u>SOLO-2, Significant improvement in PFS.</u> <u>Pivotal study, showed equivalence to AbbVie's Humira.</u>
<u>Boehringer Ingelheim GMBH</u>	biosimilar adalimumab (BI 695501)	rheumatoid arthritis	
<u>ProMetic Life Sciences Inc. /Hematech Biotherapeutics Inc.</u>	plasma-derived plasminogen	plasminogen deficiency	US BLA filing to start within weeks.
<u>Eli Lilly & Co.</u>	Taltz (ixekizumab)	psoriatic arthritis	SPIRIT-P2; positive results, 2017 supplemental filings.
<u>Roche /Genentech Inc.</u>	Lucentis (ranibizumab)	polypoidal choroidal	EVEREST II; an

<u>Daiichi Sankyo Co. Ltd./Eli Lilly & Co.</u>	<i>Effient</i> (prasugrel)	vasculopathy ischemic stroke	additional indication. PRASTO-1 and 2; an additional indication in Japanese patients. Rocket 4; achieves endpoint, NDA to be resubmitted.
<u>Aerie Pharmaceuticals Inc.</u>	<i>Rhopressa</i> (netarsudil) ophthalmic solution	glaucoma, ocular hypertension	ZOSTER-004; supports flexible dosing, and using with flu vaccine.
<u>GlaxoSmithKline PLC</u>	<i>Shingrix</i> vaccine	shingles	POLARIS-1,2,3,4; once daily single tablet. Met primary endpoint and well tolerated.
Gilead Sciences Inc.	sofosbuvir, velpatasvir and voxilaprevir	hepatitis C	
<u>Theravance Biopharma Inc. /Mylan NV</u> Phase III Initiated	revefenacin	chronic obstructive pulmonary disease	
<u>Alexion Pharmaceuticals Inc.</u>	ALXN1210	atypical hemolytic uremic syndrome and paroxysmal nocturnal hemoglobinuria	A longer-acting anti-C5 antibody, given every eight weeks.
<u>Maruishi Pharmaceutical Co. Ltd./Faron Pharmaceuticals Oy</u>	<i>Traumakine</i> (FP-1201-lyo)	acute respiratory distress syndrome	A European study is already underway.
<u>Ultragenyx Pharmaceutical Inc. /Kyowa Hakko Kirin Co. Ltd.</u>	KRN23	pediatric X-linked hypophosphatemia	A fully human IgG1 Mab.
<u>Protalix BioTherapeutics Inc.</u>	PRX-102	Fabry disease	A modified human alpha-GAL-A protein. The CanStem303C study.
<u>Sumitomo Dainippon Pharma Co. Ltd.</u> Phase II Suspended	napabucasin	colorectal cancer	
<u>Aduro Biotech Inc.</u>	CRS-207, ADU-623	ovarian, brain, and pancreatic cancer	<u>FDA partial clinical hold due to Listeria infection.</u>
GlaxoSmithKline PLC	losmapimod	chronic obstructive pulmonary disease	Development stopped by company.
Phase II Results			
<u>Minerva Neurosciences Inc. /Mitsubishi Tanabe Pharma Corp.</u>	MIN-101	schizophrenia, negative symptoms	Six-month extension study; improved symptoms.
<u>ChemoCentryx Inc.</u>	avacopan (CCX168), a	C3 glomerulopathy	A patient with this rare

	complement inhibitor		disease responded.
<i>Shire PLC</i>	maribavir	CMV infection	Reduced viremia in transplant patients.
<i>Trevena Inc.</i>	oliceridine	pain	Safe and effective.
<i>Bristol-Myers Squibb Co.</i>	<i>Opdivo</i> (nivolumab)	heavily pretreated classical Hodgkin's lymphoma	CheckMate-205; safe and effective in an expanded cohort.
Phase II Completed			
Samumed	SM04554	androgenetic alopecia	Safe and effective.
Phase II Interim/top-Line Results			
<i>OncoGenex Pharmaceuticals Inc./Ionis Pharmaceuticals Inc.</i>	apatorsen; an antisense heat shock protein 27 production inhibitor	metastatic bladder cancer	Borealis-2; positive survival results.
<i>Sanofi/Lexicon Pharmaceuticals Inc.</i>	sotagliflozin	type 1 diabetes	inTandem4; confirms Phase III dose.
<i>Windtree Therapeutics Inc.</i>	<i>Aerosurf</i> (lucinactant)	respiratory distress syndrome	In premature infants.
<i>ObsEva SA</i>	nolasiban (OBE001); oral oxytocin antagonist	in vitro fertilization	IMPLANT; proceeding to Phase III in Europe.
<i>Galapagos NV/AbbVie Inc.</i>	GLPG1837	cystic fibrosis	SAPHIRA 2; well tolerated
<i>Allergan PLC /Motus Therapeutics Inc.</i>	relamorelin, a ghrelin agonist	diabetic gastroparesis	Safe and effective, in diabetes patients.
<i>Advaxis Inc.</i>	axalimogene filolisbac	cervical cancer	Increased overall survival rate
<i>DBV Technologies SA</i>	<i>Viaskin Peanut</i>	peanut allergy	OLFUS-VIPES; two year follow-up showed durable responses.
		non-alcoholic steatohepatitis (NASH),	
Gilead Sciences Inc.	selonsertib (GS-4997)	pulmonary arterial hypertension, diabetic kidney disease	Showned antifibrotic activity.
Phase II Initiation			
<i>Targovax ASA</i>	ONCOS-102/GM-CSF gene therapy	malignant mesothelioma	Increases tumor infiltrating lymphocytes.
<i>Selecta Biosciences Inc.</i>	SEL-212	gout	In refractory patients.

Stealth BioTherapeutics	elamipretide	congestive heart failure	The IDDEA-HF study.
<u>Actelion Pharmaceuticals Ltd.</u>	ACT-541468	insomnia	In adult patients.
Theranexus	THN102	narcolepsy	Being compared with modafinil.
Sanifit	SNF472	calciphylaxis	An orphan disease.

Source: Source: Informa Pharma Intelligence's Biomedtracker.

Marketing Approvals – October 21 to October 27, 2016

Lead Company	Partner Company	Drug	Indication	Market	Comments
REGULATORY APPROVAL					
Merck & Co	-	Zinplava (bezolotoxumab)	recurrence of Clostridium difficile-associated diarrhea	US	<u>In patients receiving antibacterial drugs.</u>
<u>Recordati Industria Chimica & Farmaceutica SPA</u>	Apricus Biosciences Inc.	Vitaros (alprostadil) cream	erectile dysfunction	Greece	Launch expected in first half of 2017.
SUPPLEMENTAL REGULATORY APPROVAL					
Merck & Co	-	Keytruda (pembrolizumab)	first line non-small cell lung cancer	US	<u>For tumors with a high PD-L1 expression and no EGFR or ALK tumor aberrations.</u>
<u>Novartis AG</u>	-	Cosentyx (secukinumab)	psoriatic arthritis, ankylosing spondylitis	Switzerland	Targets IL-17.

Source: Biomedtracker.