

Pharmasset continues to lead small cap gainers so far in 2011



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High hopes for Pharmasset's hepatitis C pipeline showed no sign of fading over the last three months, the impressive near quadrupling in stock market value notched up since the beginning of the year makes the US company the best performing small cap nine months into 2011, *EvaluatePharma* data show.

Demand for Jazz Pharmaceuticals and Pharmacyclics has also remained high, with both companies' stock doubling in value so far this year, while among the micro-caps Endocyte has emerged as a rare IPO success story - shares in the university spinout have advanced 77% since floating in February. On the flip side this year has also seen its fair share of casualties, with the likes of Diamyd, Bavarian Nordic, Intercell and NeurogesX heading toward a disastrous year on the stock market.

Into the big league

With a market value of more than \$6bn, Pharmasset looks a likely graduate to the mid-cap league next year. The release of abstracts on the last day of the third quarter, from two closely-watched trials due to be presented at an approaching liver disease conference, caused the stock to set another record closing price, of \$82.37.

The weight of substantial expectations rests largely on the shoulders of the company's most advanced agent, the nucleotide analogue polymerase inhibitor PSI-7977, which is still only in phase II studies. Positive data need to keep flowing for the company to retain this massive valuation.

Almost as impressive this year has been the spectacular resurrection of Jazz Pharmaceuticals; the company's market value has more than doubled to \$1.75bn this year on hopes for cataplexy drug Xyrem. The company sought to allay fears it was essentially a one-trick pony last month through the merger with fellow specialty player Azur Pharma, a well-received deal that prompted further share price gains.

Pharmacyclics has also fared well on the back of hopes for a single product, the kinase inhibitor PCI-32765, which is still undergoing mid-stage, phase II studies. As a rare inhibitor of the Bruton's tyrosine kinase, and almost certainly the most advanced in development, hopes for a lucrative licensing deal have been driving interest in the stock.

Questcor meanwhile has been riding high on buoyant demand for Acthar, a treatment for the exacerbations of multiple sclerosis. Like Jazz with Xyrem, the company has benefited from gaining orphan status for the product, facilitating significant drug price hikes.

Finally among the small cap risers, Mesoblast has had a good year since signing a significant licensing deal with Cephalon in the final days of 2010, and on encouraging data from its stem cell-based product Revascor, in treating heart disease.

Small cap (\$250m-\$2.5bn) pharma companies: top risers and fallers in 9M 2011

Rank	Top 5 Risers	Share price (local currency)			Market capitalisation (\$m)		EP Vantage comment and analysis
		31-Dec-10	30-Sep-11	% change	YE 2010	9M 2011	
1	Pharmasset (\$)	21.78	82.37	278%	1,607	6,211	Pharmasset's soaring valuation prompts pressure to deliver
2	Jazz Pharmaceuticals (\$)	19.68	41.50	111%	786	1,747	Jazz continues to produce sweet music
3	Pharmacyclics (\$)	6.08	11.83	95%	415	809	Trial success opening doors for Pharmacyclics
4	Questcor Pharmaceuticals (\$)	14.73	27.26	85%	919	1,699	Daily Market Movers (27 Jul 2011)
5	Mesoblast (AUS\$)	4.67	8.09	73%	1,300	2,445	EP Vantage Interview - Heart disease a fertile field for Mesoblast
	Top 5 Fallers						
1	Diamyd Medical (SKr)	129.50	8.95	(93%)	275	39	Market allergic to failure of Diamyd diabetes vaccine
2	Bavarian Nordic (DKr)	245.0	40.1	(84%)	564	199	Bavarian Nordic misses out on Provenge effect
3	Intercell (€)	11.60	2.05	(82%)	746	142	Intercell resets strategy as staph vaccine fails
4	Active Biotech (SKr)	169.00	31.00	(82%)	1,629	334	Laquinimod falls behind MS pack
5	SIGA Technologies (\$)	14.00	3.27	(77%)	686	168	Vantage Point - Biotech partners force shifts in US biosecurity funding

The list of biggest small cap fallers reflects a pretty disastrous year so far for the Scandinavian pharmaceutical scene. As well as Diamyd, Bavarian and Active Biotech, NeuroSearch and Swedish Orphan Biovitrum have also registered big losses this year and rank just outside the top five presented here.

The failure of Active Biotech's oral MS drug, laquinimod, in pivotal trials has been compounded by the astonishing performance of Biogen Idec's oral MS candidate BG-12. Diamyd was forced to scrap further development of its diabetes vaccine after a European trial failed to show significance and partner Johnson & Johnson handed back its rights to the product.

Investor sentiment towards Bavarian and Intercell has nose-dived this year as both companies struggle with clinical setbacks and doubts about their strategic direction.

SIGA Technologies features for the first time this year, its reliance on government contracts for the supply and research of its biosecurity franchise making its share particularly volatile. A US government investigation in June into how the company secured a \$2.8bn smallpox treatment contract started to worry investors, compounded by the recent disappointment of a litigation battle which ruled that SIGA must share 50% of its profits with PharmAthene from a separate smallpox contract potentially worth \$433m.

Micro cap analysis

Endocyte tops the micro cap risers once more. Although its shares started to fall back a bit in the third quarter, the stock continues to trade significantly higher than its public debut in February. The go-ahead in April to file for accelerated European approval for its advanced ovarian cancer treatment and companion diagnostic was a critical milestone for the company.

Curis also features again, phase II success earlier this year in advanced skin cancer with its hedgehog signalling pathway inhibitor led to partner Roche recently filing for accelerated approval.

Meanwhile Bionomics and Pacira appear for the first time, the latter company putting in a decent performance since its IPO the day before Endocyte. Encouraging clinical trial progress with vascular disrupting agent BNC105 in the treatment of various solid tumours has boosted Bionomics, while expectations have gradually been rising for FDA approval of Pacira's reformulated analgesic Exparel.

Micro cap (\$100m-\$250m) pharma companies: top risers and fallers in 9M 2011							
Rank	Top 5 Risers	Share price (local currency)			Market capitalisation (\$m)		EP Vantage comment and analysis
		31-Dec-10	30-Sep-11	% change	YE 2010	9M 2011	
1	Endocyte (\$)	6.00	10.60	77%	178	377	No post-IPO blues for Endocyte
2	Curis (\$)	1.98	3.16	60%	135	242	Therapeutic focus - Hedgehog targets given boost with phase II skin cancer data
3	Bionomics (AUS\$)	0.32	0.48	50%	110	178	Daily Market Movers (12 Apr 2011)
4	Pernix Therapeutics (\$)	6.05	8.82	46%	137	227	EP Vantage Interview - Seek and Pernix offer up new cough drug
5	Pacira Pharmaceuticals (\$)	7.00	9.93	42%	121	171	Event - Pacira awaits verdict on pain medication
	Top 5 Fallers						
1	NeurogesX (\$)	6.36	0.89	(86%)	114	26	Product sales in 2010: hits and misses
2	Renovo (£)	0.70	0.15	(79%)	210	45	Renovo could be fatally wounded by trial failure
3	NuPathe (\$)	9.06	2.02	(78%)	132	30	FDA rejection means NuPathe has a longer path to market
4	Somaxon Pharmaceuticals (\$)	3.15	0.87	(72%)	142	41	
5	Nabi Biopharmaceuticals (\$)	5.79	1.68	(71%)	246	72	NicVax failure casts shadow over Nabi's GSK partnership and nicotine vaccines

The failure of NeurogesX's post-shingles pain product Qutenza to get off the ground continues to hurt the Californian company, while Renovo's activities are winding down following the failure of anti-scarring agent Juvista.

NuPathe, Somaxon and Nabi are newcomers to the analysis. Classical binary events came out negative for NuPathe and Nabi – the FDA rejected NuPathe's migraine patch while Nabi's nicotine vaccine failed in the first of two pivotal trials. Somaxon's insomnia agent Silenor is another in the Qutenza category of disappointing product launches, although news last night that the FDA might sanction OTC use of the drug could offer some respite.

Nano cap analysis

Among the tiddlers of drug development, Ampio and Oncothyreon have shown encouraging signs of growth. Ampio was boosted by phase III results from a premature ejaculation drug, Zertane, while Oncothyreon has been rising ahead of phase III results, from Merck KGaA-partnered lung cancer vaccine, Stimuvax.

Trius Therapeutics appears to have risen thanks to the buzz around the IV and oral formulations of its MRSA drugs, TR-701 IV and TR-701 oral. Earlier this year Bank of America Merrill Lynch initiated coverage of the group with an \$11 price target citing the potency and potential rapid uptake of the drug, on which phase III data should emerge early next year.

Operating in the particularly tough sector of diabetic foot ulcers, Derma Sciences presented encouraging phase II data for its candidate DSC127 which enabled it to raise \$26m which should cover the cost of planned phase III trials.

Among the fallers, clinical failure is largely responsible for taking scalps, although RNAi researcher Marina Biotech has suffered as hopes for the potential of its field of specialism have dimmed.

Swiss therapeutic vaccine researcher Cytos has all but wound down after a series of pipeline setbacks and Soligenix sank after trials of its lead pipeline hope, orBec for Graft-versus-Host disease, were halted by safety monitors.

Nano cap (\$25m-\$100m) pharma companies: top risers and fallers in 9M 2011

Rank	Top 5 Risers	Share price (local currency)			Market capitalisation (\$m)		EP Vantage comment and analysis
		31-Dec-10	30-Sep-11	% change	YE 2010	9M 2011	
1	Ampio Pharmaceuticals (\$)	2.40	6.65	177%	41	191	Weekly Market Movers (to 27 May 2011)
2	Oncothyreon (\$)	3.26	5.98	83%	98	251	
3	MethylGene (C\$)	0.14	0.24	71%	45	80	Asco EventAnalyzer - 2011's winners and losers
4	Trius Therapeutics (\$)	3.71	6.30	70%	88	180	
5	Derma Sciences (\$)	4.70	7.79	66%	31	82	Therapeutic Focus - Diabetic foot ulcer market in need of healing
	Top 5 Fallers						
1	Marina Biotech (\$)	1.55	0.14	(91%)	43	12	Vantage Point - Clinical progress required to reignite RNAi field
2	Curaxis Pharmaceutical (\$)	0.55	0.08	(85%)	40	6	
3	Cytos Biotechnology (SFr)	12.50	2.27	(82%)	68	15	
4	OXiGENE (\$)	4.60	1.01	(78%)	25	15	
5	Soligenix (\$)	0.19	0.04	(77%)	41	10	Daily Market Movers (15 Sep 2011)