Versartis

Equity Research

Company Quick Take

January 5, 2015

Price: $22.33 (01/2/2015)
Price Target: $45.00

OUTPERFORM (1)

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Key Data

Symbol	NASDAQ: VSAR
Market Cap (MM)	$540.3

Outstanding Data – Our Already High Level Of Conviction Is Further Strengthened

The Cowen Insight

VSAR has reported exceedingly positive VRS-317 Phase II extension study data. With the Phase III program to begin shortly, we – and our consultants – believe the positive results observed to-date indicated a high probability of success. The bottom-line is VRS-317 has the potential to be the first long-acting pediatric hGH product to reach the market, which should drive significant value creation.

Phase III Program Further De-Risked And VRS-317 Continues To Lead Long-Acting hGH Therapies In Development

Versartis provided a clinical update and reported positive data from the ongoing VRS-317 (long-acting semi-monthly growth hormone) extension study. We discuss the data release in significant detail below, but first, our key takeaways are that: (1) the 3.5 mg/kg semi-monthly dose that will be taken into Phase III resulted in IGF-1 SDS levels in the optimal, upper portion of the therapeutic range; (2) transitioning initial 1.15 mg/kg weekly patients treated for 6 months to 3.5 mg/kg semi-monthly dose for 6 months resulted in an almost 2 cm/year increase in height velocity to 9.3 cm/year after 12 months corresponding to IGF-1 increases in-line or greater than that observed with daily hGH therapies; (3) significantly less waning of height velocity was observed with VRS-317 relative to that observed with daily hGH therapies in year 1; and (4) the safety profile appears exceedingly clean and comparable to daily hGH therapies. In particular, we – and our consultants – are increasingly unconcerned by any transient excursions above IGF-1 SDS values of 2. This appears to be an investor concern, but not a concern of our consultants and exceedingly unlikely to cause any regulatory issues with the FDA (supporting information below). Overall, this additional data – in combination with the Phase II efficacy data to-date, which our consultants believe are compelling and demonstrates a safety profile comparable to currently marketed daily therapies – leads us to believe that VRS-317 leads the competitive field in long-acting hGH therapies. Given the unique long-acting dosing profile (only candidate in development with dosing greater than a week in duration), we believe that this potentially transformational program would be exceedingly attractive to various large pharmaceutical players with already established, successful daily hGH franchises. Our consultants continue to suggest that the largest unmet need with respect to current daily hGH therapies is compliance/convenience, as a large percentage of treated patients are significantly non-compliant, resulting in decreased height velocity – or more specifically – a lack of efficacy over time. The bottom line is that we – and our consultants – continue to believe that VRS-317, if successfully developed as a long-acting product, would alter the standard of care.

As for future VRS-317 milestones, the Phase III VELOCITY study in pediatric GHD patients should begin early this year with an interim data disclosure of the 6-month height velocity analysis (means and confidence intervals) anticipated in mid-2016, while topline 12-month data (primary endpoint) should be available in early 2017.

Based upon the positive Phase II and extension study data, we view the VRS-317 program as significantly de-risked as our consultants indicate that the initial efficacy profile should be highly predictive of the eventual 12 month results in the soon-to-be-initiated Phase III pivotal study. These time-points and data completion should allow VRS-317 to remain on-track for a mid-2018 FDA approval and launch – which importantly – could allow the company to be first to market with a long-acting pediatric growth hormone deficiency therapy and provide Versartis with an important first-mover advantage in a treatment area currently fragmented by undifferentiated daily treatment options. Moreover, this trial design should allow for subsequent filings in Canada and Western Europe. A monthly dose will be evaluated as part of the VRS-317 lifecycle expansion strategy and we should receive an update on that program later this year. The company has also conducted extensive discussions with the PMDA regulatory agency in Japan and plans to initiate a parallel Phase II/III pediatric trial for VRS-317 early this year with potential 12 month data by H1:2017 for the semi-monthly formulation. Finally, Versartis also expects to initiate a Phase II/III registration study for once-monthly VRS-317 in adults starting H2:15, which is also a significant additional market opportunity. Lastly, an additional undisclosed clinical development candidate could be announced around the middle of this year. The bottom-line is that Versartis is aggressively driving forward with multiple programs, should have a first-mover advantage in the long-acting market (with its semi-monthly formulation), and should eventually complement this franchise with a potential once-monthly product. The strategy is sound and the technology is differentiated, and we would be adding at these levels.

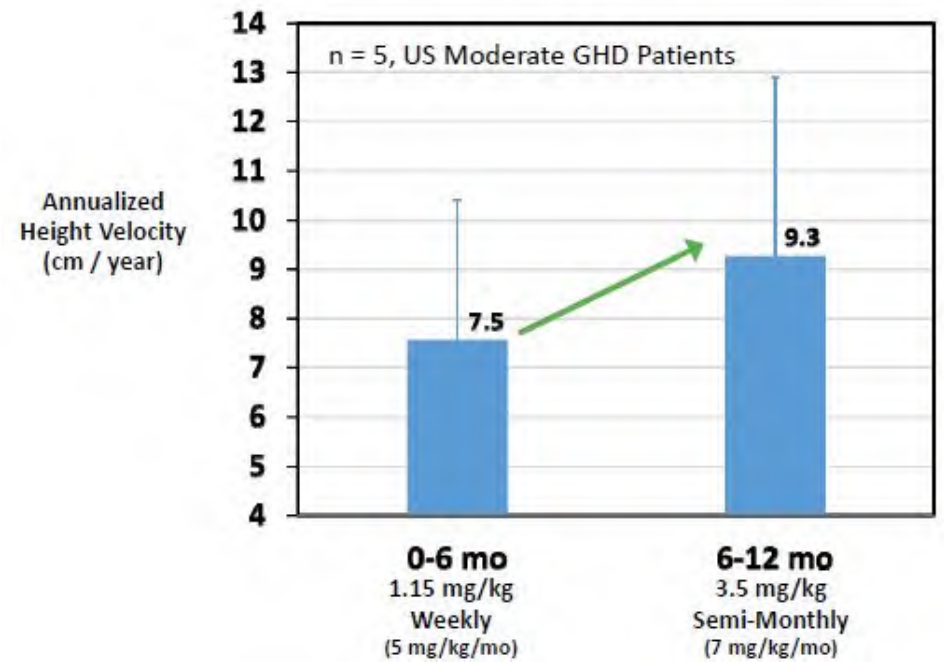
Specifics Of The Extension Study Data Update With A Focus On The New 3.5 mg/kg Semi-Monthly Dose To Be Used In Phase III

As for the specifics of the data update, 95% of the Phase IIa pediatric GHD patients (n=60/64) rolled over into an ongoing extension study, which treated patients with 2.5 mg/kg semi-monthly, 3.5 mg/kg semi-monthly (1.15 mg/kg weekly patients from Phase IIa transferred to this new dose), and 5.0 mg/kg monthly. Recall, that the newer 3.5 mg/kg semi-monthly dose will be used for the proposed Phase III VELOCITY trial in pediatric GHD patients, which was selected based upon the company's proprietary PK/PD model. Importantly, in the group of patients initially treated 6 months with 1.15 mg/kg weekly dose in Phase IIa that were then transitioned to the newer 3.5 mg/kg semi-monthly dose for 6 months (n=5), IGF-1 levels increased as predicted and were almost a full standard deviation above those observed with the prior 2.5 mg/kg semi-monthly dose. To put it another way, the 2.5 mg/kg semi-monthly regimen resulted in a mean IGF-1 SDS value of -0.4 (n=19), which is the lower portion of the therapeutic range, whereas treatment with the 3.5 mg/kg semi-monthly regimen resulted in a mean value of 0.5 (n=16), which is in the upper portion of the therapeutic range. We would note that the 0.5 mean IGF-1 SDS value is nearly identical to that observed with most common daily hGH dose of 40 ug/kg/day in almost the same patient population (based on age and height SDS demographics). Furthermore, no overexposure to IGF-1 was observed at the 3.5 mg/kg dose.

Not surprisingly, this increase in dose and corresponding mean IGF-1 SDS levels also resulted in an average increase in height velocity of nearly 2 cm/year (n=5) between the first 6 months of treatment at 1.15 mg/kg weekly (7.5 cm/year annualized) and the second 6 months of treatment at 3.5 mg/lg semi-monthly (9.3 cm/year annualized). We wonder if the height velocities above 9.3 cm/year could be achieved in patients if they are in fact treated with 3.5 mg/kg semi-monthly VRS-317 for the first full 12 months (as in the planned Phase III), as opposed to just the second 6 months per above. This seems plausible, especially considering the importance of catch-up growth in the first 6 months of initiation of treatment. Additionally, there appears to be less waning of growth response over the first year with VRS-317 when compared to daily

hGH therapies. Using annualized height velocities at 3, 6, and 12 months, daily hGH therapies declined from 10.9 cm/year to 8.6 cm/year (a decline of 2.3 cm/year over 12 months), while VRS-317 2.5 mg/kg declined from 8.9 cm/year to 8.5 cm/year (a decline of 0.4 cm/year over 12 months). We see no reason to believe that less waning shouldn't occur with the 3.5 mg/kg semi-monthly regimen as well and wonder if this impact will continue to be observed into the second year of treatment, especially as compliance with daily therapies tends to drop off. This would clearly be an exceedingly positive development if it were to occur.

VRS-317 Dosage Increase To 3.5 mg/kg Semi-Monthly Resulted In Increased Height Velocity In The Same GHD Children During Extension Study



Source: Versartis

From a safety perspective, all VRS-317 regimens, including the newer 3.5 mg/kg semi-monthly regimen, continue to be very well tolerated with the few observed AEs being very mild (grade 1) and transient. There were no drug-related SAEs, unexpected AEs, or lipoatrophy or nodules, which has been historically observed with daily hGH therapy. The number of AEs also decrease from the first to second 6 months of treatment. Lastly, 2 out of the 20 patients on the 3.5 mg/kg semi-monthly dose experienced 1 transient IGF-1 SDS excursion above 2 each. We – and our consultants – are completely unconcerned by this for the following reasons: (1) The FDA only is concerned about excursions above 3 IGF-1 SDS; (2) only excursions of chronic nature (these are transient and one-off) are concerning from a safety perspective; (3) as observed in a publication by Cohen et al. (J Clin Endocrinol Metab, July 2007, 92(7):2480–2486), even observing high doses of hGH where IGF-1 SDS levels at 2 or above were reached by 9 months and *maintained* in patients, safety did not differ from the lower, labeled doses of hGH and the incidence of AEs were similar to those reported in other studies; and (4) Nutropin AQ was approved with numerous observed excursions above an IGF-1 SDS of 2, and 3 as well, which has never been observed with VRS-317. Put simply, only chronic and maintained IGF-1 SDS levels above 3

should be of concern – these two observations and everything observed to date are not.

If approved, Versartis' semi-monthly option would reduce the current burden associated with daily hGH treatment for pediatric patients by not only reducing the number of administrations (from 365 to 24 a year), but also minimizing the complexity of the device. Daily hGH options have a multi-step administration process using a pen (needle gauge of 30-32) that in most cases needs to be refrigerated. For VRS-317, a simple autoinjector device (needle gauge of 29-30) will be utilized and can be stored at room temperature. We are unconcerned regarding this needle size and consultants suggest that it should not have any significant effect on patient use. Given the nature of GHD, Versartis plans to use a targeted specialty sales force to commercialize VRS-317. The company estimates that it can focus its efforts on the roughly 800 high prescribing pediatric endocrinologists with about 50 sales reps.

The Valuation Suggests Potential Significant Upside

As for the valuation, with the positive 6 month and extension study VRS-317 results and subsequent de-risking, we arrive at a base case valuation of $45 per share. While VSAR shares are unlikely to find their way to those levels immediately, we believe that they should approach our price target as the Phase III program progresses and we near both the 6 and 12 month data readouts – and as investors begin to better understand that the risks associated with these events are relatively low. Further de-risking in Phase III for VRS-317 and assuming commercial success should take the value closer to $65-70. We have included peak sales estimates of $400MM+, $400MM +, and approximately $250MM in the target US, EU, and Japanese pediatric GHD markets, respectively. However, we – and our consultants – believe that these peak sales estimates could likely prove conservative if the long-acting profile of VRS-317 holds up (which we believe it will) and no safety issues are observed, as use could spread into other indications. The market potential from the adult GHD indication/ program, which is also progressing, is clearly not included in these estimates.

The VRS-317 Pediatric GHD Phase III VELOCITY Program

Following interactions with the FDA and EMA, Versartis announced plans for the Phase III trial of VRS-317 in pediatric growth hormone deficient patients to begin early this year. Management has selected semi-monthly dosing – which was as expected – as the commercial formulation is already prepared to move into clinical trials. Versartis expects to initiate the global Phase III pivotal program for VRS-317 in early 2015 with up to 70 clinical sites across the U.S., Canada, and Western Europe (using many of the same sites as in Phase II). The pediatric patient demographics will be similar to the Phase II study (further de-risking the program), which enrolled moderately severe GHD patients with a mean age of 7-8. In our – and our consultants' – view, Versartis is studying VRS-317 in the appropriate patient population and these demographics are representative of typical GHD patients seen in the major markets where Versartis is focusing its commercial efforts. The study will utilize an open-label design with a non-inferiority 12 month height velocity endpoint for 3.5 mg/kg semi-monthly VRS-317 relative to current daily GHD therapy. Approximately 136 patients will be randomized 3:1 to either 3.5mg/kg semi-monthly VRS-317 or 34 µg/kg of daily rhGH, which is the highest approved dose on the labels of Genotropin and Norditropin in all regions. Of note, the company has increased the dose from the 2.5 mg/kg used in the Phase II study to 3.5mg/kg based on extensive PK/PD modeling to determine an optimal dose that will provide catch-up growth at the highest tier. Given the clean safety and efficacy profile of the relatively low doses studied in Phase II, we had previously believed Versartis had the ability to take the VRS-317 dose to higher levels and achieving greater HV results. The company has transitioned 20 VRS-317 patients from Phase II to the 3.5 mg/kg dose in the ongoing safety expansion study with no

issues, and the remaining patients will be converted by year-end. The 6-month data provided today on the patients using the 3.5 mg/kg dose provides valuable insight on safety and efficacy before heading into Phase III and further de-risks the program. Finally, Versartis is currently in the process of developing an injection device for the product based on feedback from the FDA and the majority of patients in the Phase III study are expected to receive a single injection per administration using a 30 gauge needle.

Overview Of Previously Reported Phase II VRS-317 Data

Versartis presented positive 6 month VRS-317 results from a Phase IIa pediatric GHD study at the ICE/ENDO 2014 annual conference in Chicago in late June. VRS-317 demonstrated efficacy and safety comparable to the highest approved doses of market-leading daily growth hormone therapies, Genotropin and Norditropin. Similar to the initial 3 month data, the primary endpoint of repeat weekly, semi-monthly, and monthly dosing of VRS-317 over a 6 month period showed that annualized 6 month height velocity measurements were comparable to age-matched historical controls (on daily injection). Importantly, VRS-317 was safe and well-tolerated in the naive, pre-pubertal children as only mild and transient adverse events, which are typically observed with daily hGH therapy were observed. There were no SAEs or unexpected AEs. There were 5 patients with transient excursions of IGF-I SDS scores above 2 in the 5 mg/kg monthly dosing cohort, but as we stated previously, this is un-concerning to both us and our consultants. We note that the cutoff of 2 is the company's own stringent requirement and that the FDA is more concerned about levels reaching 3 (per hGH treatment guidelines), which did not occur in the study. Also, as indicated by "transient excursions," these IGF-I SDS scores subsequently returned to normal. The bottom line is that after an additional 3 months of VRS-317 treatment exposure (up to 6 month total), VRS-317 demonstrated impressive efficacy, a safety profile that appears to be cleaner than previous long-acting therapies in development, and is leading the pack among long-acting hGH therapies in development.

Valuation Methodology And Risks

Valuation Methodology

Pharmaceuticals/Specialty

For our valuation methodology, we arrive at fair value utilizing a discounted cash flow (DCF) approach to derive our 12-month price target.

Investment Risks

Pharmaceuticals/Specialty

Risks include: (1) growing competitive dynamics in the specialty pharmaceuticals space; (2) the ability of management to execute on external growth by successfully acquiring new strategic, accretive products; (3) the ability to grow organically and keep the product pipeline robust; (4) potential regulatory delays, rejections, or failures of pipeline products; (5) economic sensitivity of any self-pay products or weakening consumer demand; (6) domestic or international pricing pressures for marketed products; and (7) failure to execute on new product launches.

Risks To The Price Target

Versartis is an early stage clinical development company with a single product and with that carries risk. Failure of Versartis to successfully develop VRS-317, for which its valuation is solely predicated on, could result in a significant decrease to its valuation and corresponding share price.

Addendum

Stocks Mentioned In Important Disclosures

Ticker	Company Name
VSAR	Versartis

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Assumption: The expected total return calculation includes anticipated dividend yield

Cowen and Company Rating System until May 25, 2013

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Assumptions: Time horizon is 12 months; S&P 500 is flat over forecast period

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Buy (a)	461	60.50%	109	23.64%
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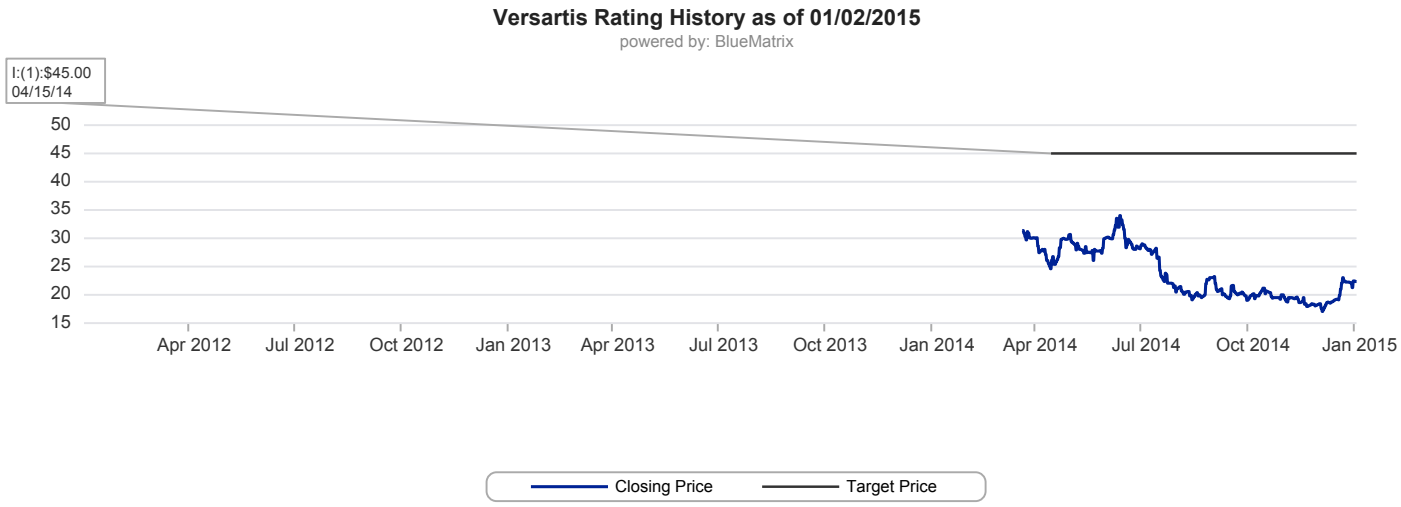

Legend for Price Chart:

I = Initiation | 1 = Outperform | 2 = Market Perform | 3 = Underperform | UR = Price Target Under Review | T = Terminated Coverage | $xx = Price Target | NA = Not Available | S=Suspended

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