EDISON

Achillion Pharmaceuticals

Commercialisation update

From takeout to taking it on

Healthcare equipment & services

Achillion's collaborative deal with Janssen Pharmaceuticals (Johnson and Johnson), announced 19 May, repositions Achillion as a viable, independent concern with a high-potential Hep-C franchise, a small molecule programme targeting the complementary Factor D pathway and noteworthy research platform. The agreement lays to rest any speculation as to an outright company takeover. The deal, entailing up to $900m in milestones and a $225m equity investment, is value enhancing on our analysis, moving our valuation to $2.3bn from $1.9bn as Janssen takes on all R&D costs, paying royalties on sales from mid-teens to low twenties.

Year end	Revenue ($m)	PBT* ($m)	EPS* ($)	DPS ($)	P/E (x)	Yield (%)
12/13	0.0	(53.0)	(0.56)	0.0	N/A	N/A
12/14	0.0	(61.7)	(0.63)	0.0	N/A	N/A
12/15e	0.0	(52.7)	(0.44)	0.0	N/A	N/A
12/16e	0.0	(48.0)	(0.35)	0.0	N/A	N/A

Note: *PBT and EPS are normalised, excluding intangible amortisation, exceptional items and share-based payments.

Deal with Janssen (J&J) de-risks Hep-C development

Janssen has acquired the rights to develop and commercialise ACH-3102 (NS5A inhibitor), ACH-3422 (nuc) and sovaprevir (protease inhibitor) with the intention of developing a short-duration, highly effective, pan-genotypic Hep-C treatment that would potentially be best-in-class. Achillion's agreement with Janssen brings an experienced and skilled development and commercialisation partner, while freeing up valuable financial resources to support its budding earlier-stage research programme. Additionally, Janssen brings its own portfolio of potential Hep-C candidates to the deal, mitigating risk of clinical failure and providing the potential for acceleration of the development timeline. We await next news in the coming months on the structure and timeline of the new development programme.

IND submission of Factor D compound later this year

Achillion confirms that its discovery programme for novel oral small molecule inhibitors of complement factor D (fD) is on track and that an IND for a lead compound will be submitted by year end. Focus thus far has been on paroxysmal nocturnal hemoglobinuria (PNH), a rare, life-threatening blood disorder. However, potential in indications with considerably larger patient populations, including dry age-related macular degeneration (AMD), myasthenia gravis and atypical haemolytic uremic syndrome (aHUS) are also being explored.

Valuation: Moves up to $2.3bn from $1.9bn

Our valuation of $2.3bn ($16.7 per share or $16.1 per diluted share) moves up from $1.85bn ($15.8 per share or $15.1 per diluted share) primarily on value enhancements connected to the collaboration with Janssen, which includes $1.1bn in new equity and potential milestones. We estimate current total cash at ~460m (including the equity investment), which should be sufficient to take Achillion through to profitability on the planned launch of its Hep-C combination regimen in 2018.

21 July 2015

Price	**US$9.36**
Market cap	**US$1101m**

Net cash* ($m) at March 2015) *Before $225m equity investment by Janssen	275.9
Shares in issue	117.6m
Free float	88%
Code	ACHN
Primary exchange	NASDAQ
Secondary exchange	N/A

Share price performance

%	1m	3m	12m
Abs	6.5	(6.9)	37.2
Rel (local)	5.6	(8.1)	27.6
52-week high/low	US$15.99		US$6.71

Business description

Achillion is a biopharmaceutical company engaged in the discovery and development of treatments for chronic hepatitis C virus (HCV) and other therapeutic areas. The company is collaborating with Janssen Pharmaceuticals (J&J) to develop and commercialise its Hep-C franchise including NS5A inhibitor ACH-3422, a novel nucleotide NS5B polymerase inhibitor, ACH-3102, and NS3/4A protease inhibitor, sovaprevir.

Next events

H115 results	August 2015
Revised Hep-C development plan	H215
Start of Factor D Phase I	H215

Analysts

Katherine Genis +1 646 653 7026

Christian Glennie +44 (0)20 3077 5727

healthcare@edisongroup.com

Edison profile page

Company adaptation: Transformative collaboration

Achillion's recently signed collaboration with Janssen Pharmaceuticals (J&J) secures its spot as an independent and ongoing concern focused on the build-out of its own research platform. The market penalised the Achillion stock by 18% on 19 May potentially due to disappointment that it was not being directly acquired. However, our revised model indicates that the Janssen agreement adds considerable value to the company. We believe further communication about the newly shaped Hep-C development programme and further attention to the mechanics of the deal, including its significant milestone payments, will contribute to a steadily renewed appreciation of the newly shaped firm by investors.

Janssen to take on development and commercialisation

On 19 May, Achillion announced that Janssen had acquired worldwide licensing rights to develop and commercialise three Hep-C drugs in the Achillion portfolio: ACH-3102 (NS5A inhibitor), ACH-3422 (nuc) and sovaprevir (protease inhibitor). The agreement was formed by the two companies with the intention of developing a short-duration, highly effective, pan-genotypic Hep-C treatment that would potentially be best-in-class.

Under the terms of the agreement, the initial triple regimen to be developed will include Achillion's NS5A inhibitor with an NS3/4A protease inhibitor and NS5B nuc. The exact compounds of the last two have yet to be decided, although both Achillion and Janssen have candidates in each category. Janssen brings two additional Hep-C candidates to the table for inclusion in the potential triple regimen: NS5B polymerase inhibitor ALS-335 and the already approved NS3/4A protease inhibitor (PI), Olysio. As indicated by the companies, Olysio's status as a currently marketed PI could allow for an accelerated time to commercialisation for the triple regimen (and Olysio is therefore likely to be pursued as a component in the mix). Olysio was previously heavily prescribed with Gilead's Sovaldi before Gilead's launch of Harvoni late last year. We await news in the coming months on the structure and timeline of the new development programme and drugs to be developed in the regimen.

As announced by the companies, Janssen will wholly fund all development and commercialisation costs, while Achillion is eligible for more than $900m in development, regulatory and commercial milestones. Royalties on sales will be made on a sliding scale from mid-teens to low twenties. Notably, both milestones and royalties will be paid on any regimen containing any one Achillion compound. In lieu of an upfront payment, Janssen, through its investment vehicle, Johnson and Johnson Innovation (JJDC) is investing $225m in 18.4m new Achillion shares at a price of $12.25 per share or a 14.7% premium on Achillion's stock, which traded at $10.68 per share on the morning of the announcement. In our view Achillion, together with Janssen, has increased its chances of marketing the first triple combination – NS5A, NS3 and nucleotide polymerase –and best in class Hep-C treatment. The link-up with Janssen serves to firm up Achillion's positioning in Hep-C by adding two additional candidates, thereby providing the proverbial multiple shots on goal.

We view the recently signed collaboration with Janssen Pharmaceuticals as transformative for Achillion. With a clear path towards commercialisation of its oral Hep-C compound, Achillion is now firmly established as a standalone company with the intent to see its Hep-C combination treatment through to development and worldwide commercialisation vis-à-vis its partner. Janssen's equity purchase has helped provide the financial muster needed for the company to invest in its earlier-stage small molecule therapeutics research platform, with its complement Factor D programme at the forefront.

Exhibit 1: Hep-C triple regimens NS5A+nuc+PI

Achillion and competitor triple combination regimens

	NS5A inhibitor	NS5B polymerase inhibitor (nuc)	NS3/4A protease inhibitor
Achillion with Janssen	ACH-3102 (A)	ACH-3422 (A) ALS-335 (J)	Sovaprevir (A) Olysio (J)
Gilead	Ledipasvir GS-5816	Sofosbuvir	GS-9857
Merck	MK-8408	MK-3682	Grazoprevir

Source: Achillion Pharmaceuticals

Factor D programme moving forward

At the time of its analyst conference call on 19 May, Achillion's management reported good headway on its oral Factor D inhibitor programme, which targets complement-mediated diseases. Now evaluating compounds in preclinical and IND-enabling studies, the company confirmed plans to submit an IND regulatory package with a lead drug by the end of 2015. Factor D research has focused on paroxysmal nocturnal hemoglobinuria (PNH), for which the only approved treatment is Alexion's Soliris (2014 sales $2.2bn), which has significant limitations including intravenous administration and a black box warning of serious meningococcal infections. Achillion's research in Factor D is also ongoing in indications with considerably larger patient populations, including dry age-related macular degeneration (AMD), myasthenia gravis and atypical haemolytic uremic syndrome (aHUS).

Exhibit 2: Complement-mediated diseases

	Characteristics	Epidemiology (prevalent population US/EU-5)	Current treatment (approved and/or supportive care)
PNH 1,4	Acquired: rare from of hemolytic anemia	8,000-10,000	Eculizumab RBC transfusion
aHUS1-3	Commonly genetic: thrombocytopenia	2,000-3,000	Eculizumab RBC transfusion
Myasthenia gravis 5,6	Autoimmune: neuromuscular junction (NMJ) destruction and muscle weakness	80,000-100,000	Cholinesterase inhibitors, steroids, immunosuppression
Dry AMD (geographic atrophy, GA) 1,3,7-10	Complex multifactorial disease; loss or atrophy of retinal pigment epithelium	2,000,000-3,000,000	No treatments (prophylactic only)

Source: 1. Meri & Jarva, eLS; 2013; 2. Lorait & Fremeaux-Bacchi Orphanet. *J Rare Dis;* 2011;9:60; 3. Markiewski & Lambris . *Am J Pathol*; 2007; 71(3):715-27; 4. Hillmen, et al. *N Engl J Med*; 1995;333(19):1253-8; 5. Huda, et al. *Rev Neurosci;* 2014;25(4):575-83; 6. Kusner & Kaminski. *Ann N Y Acad Sci*; 2012;1274(1):127–132; 7. Rudnicka, et al. *Ophthalmology*; 2012;119(3):571-80; 8. Weber, et al. *Dtsch Arztebl Int;* 2014;111(8):133-8; 9. Singer M. F1000Prime Rep; 2014;6:29; 10. Loyet, et al. *J Pharmacol Exp Ther*; 2014. pii: jpet.114.215921.

The Factor D programme was initiated in house through Achillion's own research platform and represents the potential of its internal research technology beyond that of Hep-C. Newly shored-up funds on the back of the company's recent share offer and an equity infusion from Janssen enable adequate cash to further the Factor D programme well into clinical development. We expect market recognition of Achillion's early-stage capabilities in the development of novel small molecule therapeutics on successful progression of both the Hep-C and Factor D programmes.

Valuation

We increase our valuation to $2.3bn ($16.7 per share or $16.1 per diluted share) from $1.85bn ($15.8 per share or $15.1 per diluted share) as published in our last update note on 29 April 2015. The increase in valuation is due to changes in our model on the back of the collaboration with Janssen, as noted below. This is only slightly offset by a downward revision of our expected price per treatment for an Achillion/Janssen Hep-C combination from $45 to $40, which seems prudent given recent trends in price discounting by Abbvie and Gilead for their currently marketed Hep-C compounds.

We have not included a valuation in our model for Achillion's Factor D programme. However, we note that an IND is planned for Achillion's first Factor D compound by year end, which would trigger inclusion of the programme in our forecasts and valuation. This would add a nominal approximate $80m to our valuation after risk adjustments assuming peak sales of $2bn for products in paroxysmal nocturnal hemoglobinuria (PNH) and a 10% probability of success.

Basis for the valuation

Key components of our change in valuation are as follows:

- Profit from Hep-C revenue revised from a prior assumed 40% margin – originally derived from a co-development share of a gross margin to royalties on sales. Achillion will now receive potential royalties on Hep-C sales from Janssen on a sliding scale from 12%-24% as per the terms of the agreement.
- Number of shares increases on Janssen's equity purchase.
- Additional cash of $225m from Janssen's equity purchase is added.
- Our risk factor for Hep-C is increased from 45% to 60% probability of success on the addition of Janssen's drug candidates to the potential triple combination, including the already approved Olysio.
- R&D costs taken on by Janssen for Hep-C, estimated at an NPV of ~US$300m, have been removed.
- Price per treatment is lowered to $40 from $45 per treatment to reflect current trends for Hep-C products.

We await details, including timelines for the new Hep-C development programme, at which time we expect there may be scope to push our Hep-C forecasts forward on a quicker launch schedule. We currently model first sales of a potential triple Hep-C regimen in 2018, although an earlier launch schedule would edge our fair value calculation upward.

Exhibit 3: Achillion valuation

Product	Main indication	Status	Prob. of success	Launch year	Peak sales ($m)	Patent protection	Royalty	rNPV
All oral combo	US, HCV	Phase II	60%	2018	$1,764	2032	12%-24%	$572
All oral combo	EU Big-5, HCV	Phase II	60%	2018	$1,074	2032	12%-24%	$347
All oral combo	Japan, HCV	Phase II	60%	2018	$556	2032	12%-24%	$328
R&D								$558
Total								$1,806
Current cash (est post Janssen equity stake) ($m)								$460.0
Total firm value ($m)								$2,266
Total basic shares (m)								136
Value per basic share ($)								$16.7
Stock options(3/2015, m)								9.1
Total number of shares								144.8
Diluted value per share ($)								$16.1

Source: Edison Investment Research

Financials

Achillion announced its Q115 results in May, reporting a net loss of $19.3m vs a loss of $16.1m in the first quarter of 2014, which was broadly in line with our expectations. R&D spend was $15.2m in Q115 ($12.8m in Q114).

The company ended its first quarter with cash of $275.9m. We expect that current cash holdings estimated at ~$250m, together with the $225m from the equity contribution from Janssen and potential future development milestones, should be sufficient to bring the company through to profitability on the launch of its Hep-C combination in 2018. We project annual net losses of $52.7m and $48.0m in 2015 and 2016 respectively, down considerably from our previous forecast losses of $108m and $112m as Janssen will take over R&D costs related to the development work in Hep-C. At the time of full year reporting, management guided toward full year R&D costs of ~$90m, or roughly $25m per quarter, with the majority of these costs related to the Hep-C programme. We now trim our R&D forecasts by roughly 50% starting in Q315. Costs stripped out of R&D from Hep-C more than compensate for stepped-up R&D expenses now included for the initiation of the Factor D clinical programme.

Exhibit 4: Financial summary

($000)	2011	2012	2013	2014	2015e	2016e
Year end 31 December	IFRS	IFRS	IFRS	IFRS	IFRS	IFRS
PROFIT & LOSS						
Revenue	247	2,607	0	0	0	0
Cost of Sales	0	0	0	0	0	0
Gross Profit	247	2,607	0	0	0	0
EBITDA	(41,031)	(42,953)	(53,157)	(61,664)	(58,895)	(59,680)
Operating Profit (before amort. and except.)	(41,358)	(43,361)	(53,556)	(62,153)	(59,434)	(60,269)
Intangible Amortisation	0	0	0	0	0	0
Exceptionals	0	0	0	0	0	0
Stock options	(2,989)	(3,932)	(5,920)	(7,273)	(7,273)	(7,273)
Operating Profit	(44,347)	(47,293)	(59,476)	(69,426)	(66,707)	(67,542)
Net Interest	141	166	529	418	6,700	12,250
Profit Before Tax (norm)	(44,206)	(43,195)	(53,027)	(61,735)	(52,734)	(48,019)
Profit Before Tax (FRS 3)	(44,206)	(47,127)	(58,947)	(69,008)	(60,007)	(55,292)
Tax	0	0	0	0	0	0
Profit After Tax (norm)	(41,217)	(43,195)	(53,027)	(61,735)	(52,734)	(48,019)
Profit After Tax (FRS 3)	(44,206)	(47,127)	(58,947)	(69,008)	(60,007)	(55,292)
Average Number of Shares Outstanding (m)	64.25	73.97	93.98	98.37	121.30	135.90
EPS - normalised (c)	(64.2)	(58.4)	(56.4)	(62.8)	(43.5)	(35.3)
EPS - normalised and fully diluted (c)	(64.2)	(58.4)	(51.4)	(56.5)	(40.4)	(33.1)
EPS - (IFRS) (c)	(68.8)	(63.7)	(62.7)	(70.2)	(49.5)	(40.7)
Dividend per share (c)	0.0	0.0	0.0	0.0	0.0	0.0
Gross Margin (%)	100.0	100.0	N/A	N/A	N/A	N/A
EBITDA Margin (%)	N/A	N/A	N/A	N/A	N/A	N/A
Operating Margin (before GW and except.) (%)	N/A	N/A	N/A	N/A	N/A	N/A
BALANCE SHEET						
Fixed Assets	1,009	1,503	1,344	1,780	2,229	2,786
Intangible Assets	0	0	0	0	0	0
Tangible Assets	994	1,247	1,265	1,726	2,229	2,786
Restricted cash	152	152	152	152	152	152
Investments	15	256	79	54	0	0
Current Assets	81,469	79,875	160,921	154,875	460,404	423,192
Inventory	0	0	0	0	0	0
Accounts recievable, net	103	277	480	95	145	195
Cash and cash equivalents	79,943	77,418	157,989	152,879	458,058	420,496
Other	1,423	2,180	2,452	1,901	2,201	2,501
Current Liabilities	(8,944)	(9,136)	(9,403)	(13,059)	(13,134)	(13,209)
Creditors	(8,803)	(8,786)	(9,112)	(12,864)	(12,964)	(13,064)
Short term borrowings	(141)	(350)	(291)	(195)	(170)	(145)
Long Term Liabilities	(229)	(347)	(56)	(279)	0	0
Long term borrowings	(229)	(347)	(56)	(279)	0	0
Other long term liabilities	0	0	0	0	0	0
Net Assets	73,305	71,895	152,806	143,317	449,499	412,768
CASH FLOW						
Operating Cash Flow	(36,268)	(46,700)	(54,165)	(55,942)	(58,133)	(58,918)
Net Interest	132	166	529	418	6,700	12,250
Tax	0	0	0	0	0	0
Capex	(732)	(656)	(408)	(947)	(1,042)	(1,146)
Acquisitions/disposals	0	0	0	0	0	0
Financing	61,663	44,235	133,951	52,264	357,100	9,618
Net Cash Flow	24,795	(2,955)	79,907	(4,207)	304,626	(38,196)
Opening net debt/(cash)	(55,035)	(79,725)	(76,873)	(157,794)	(152,557)	(458,041)
HP finance leases initiated	0	0	0	0	0	0
Other	(105)	103	1,014	(1,030)	858	658
Closing net debt/(cash)	(79,725)	(76,873)	(157,794)	(152,557)	(458,041)	(420,503)

Source: Company accounts, Edison Investment Research

Frankfurt +49 (0)69 78 8076 960
Schumannstrasse 34b
60325 Frankfurt
Germany

London +44 (0)20 3077 5700
280 High Holborn
London, WC1V 7EE
United Kingdom

New York +1 646 653 7026
245 Park Avenue, 39th Floor
10167, New York
US

Sydney +61 (0)2 9258 1161
Level 25, Aurora Place
88 Phillip St, Sydney
NSW 2000, Australia

Wellington +64 (0)48 948 555
Level 15, 171 Featherston St
Wellington 6011
New Zealand