EDISON

Basilea Pharmaceutica

Corporate update

The future looks bright

Pharma & biotech

Basilea's prognosis looks good. Multiple licensing/distribution agreements announced for launched assets Cresemba and Zevtera should drive top-line growth faster than we had expected. Major deals include; Cresemba in Europe (ex Nordics), Russia, Turkey and Israel with Pfizer (CHF70m upfront plus up to $427m in regulatory and sales milestones and mid-teen royalties), Zevtera in China with CR Gosun, and in Europe with Cardiome. These licensing and distribution deals validate the commercial potential for both assets and enable the financial fire power to bolster Basilea's existing, innovative R&D pipeline (with a focus on cancer and anti-infective drug resistance). Our upgraded valuation is CHF1,188m or CHF110/share (excluding treasury shares) vs CHF1,048 previously.

Year end	Revenue (CHFm)	PBT* (CHFm)	EPS* (CHF)	DPS (CHF)	P/E (x)	Yield (%)
12/15	52.8	(61.3)	(6.07)	0.0	N/A	N/A
12/16	66.0	(50.9)	(5.06)	0.0	N/A	N/A
12/17e	92.4	(28.6)	(2.83)	0.0	N/A	N/A
12/18e	82.2	(17.7)	(1.75)	0.0	N/A	N/A

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

2017 a successful year for partnerships

Basilea has announced multiple licensing/distribution agreements for key assets Cresemba (isavuconazole) for invasive mould infections and Zevtera/Mabelio (ceftobiprole) for bacterial infections. The significant licensing deal with Pfizer for Cresemba (in Europe ex Nordics, Russia, Turkey and Israel) makes strategic and commercial sense given its expertise in this niche therapeutic indication. As a result we have upgraded our global Cresemba peak sales forecasts to $0.8bn from $0.6bn; the bulk of the upgrade relates to the European opportunity.

Financial capital available to focus on the next wave

With Cresemba and Zevtera's commercialisation largely in the hands of partners, Basilea can turn its focus to its next pillars of growth: notably, the initiation of Zevtera's Phase III US clinical trial programme in two new indications (we forecast US peak sales of $317m in 2027), investing in the early to mid-stage products BAL101553 and BAL3833 targeting cancer resistance and bolstering the oncology and infectious disease pipeline further through in licensing.

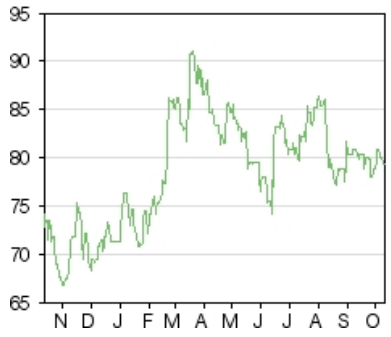
Valuation: rNPV of CHF1,188m or CHF110/share

Our revised valuation of CHF1,188m, from CHF1,048m, reflects mainly changes to our Cresemba assumptions, as a result of increased non-US sales expectations. Basilea will benefit from royalties and milestones on Cresemba sales, and we have reduced the Cresemba related COGS and SG&A expense accordingly. Our valuation is based on an NPV of the main portfolio of products and net cash. Cresemba, based on $0.8bn peak sales, is worth CHF913.5m. We also include Zevtera in Europe/ROW including the deals with Cardiome and CR Gosun, in addition to risk-adjusted contributions for the US and the earlier-stage pipeline.

11 October 2017

Price	**CHF79.25**
Market cap	**CHF936m**
	US$1.03/CHF
Net cash (CHFm) at 30 June 2017	57.3
Shares in issue (including 1m treasury shares)	11.8m
Free float	91.46%
Code	BSLN
Primary exchange	SIX
Secondary exchange	N/A

Share price performance



%	1m	3m	12m
Abs	(2.0)	(0.6)	5.6
Rel (local)	(5.8)	(4.1)	(6.9)
52-week high/low	CHF91.0	CHF66.7	

Business description

Basilea Pharmaceutica is focused on anti-infectives and oncology. Its lead products are Cresemba (an antifungal), which is approved in the US and Europe, and Zevtera (an anti-MRSA broad-spectrum antibiotic), approved in many European and non-European countries for pneumonia. Its R&D pipeline includes two clinical-stage assets for cancer resistance.

Next events

Zevtera initiate Phase III ABSSSI US study	Q417
Zevtera initiate Phase III SAB US study	H118
FY17 results	February 2018

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Edison profile page

Investment summary

Company description: Focus on resistance

Basilea Pharmaceutica is a Switzerland-based biopharmaceutical company with a focus on developing innovative antibiotics, antifungals and oncology drugs that target drug resistance and non-responsiveness to available drug treatments. Basilea has two approved hospital products, Cresemba and Zevtera, for the treatment of severe mould and bacterial infections, respectively. Both products have a broad spectrum of activity, are suitable for empiric use in the hospital setting and have advantages over current treatments. Cresemba is being commercialised in the US by partner Astellas and in the majority of European markets by Pfizer. Numerous partnerships are in place for both assets outside these markets (including Grupo Biotoscana (GBT), Unimedic Pharma, Avir Pharma, and Hikma), for Zevtera in Europe (Cardiome and China (CR Gosun). Basilea was spun out of Roche in 2000 and to date has raised around CHF925m net (including initial funding from Roche, a private placement, an IPO, a rights offer and a convertible bond issue). Basilea's headquarters are in Basel and it employs c 230 people. *

Valuation: Risk-adjusted NPV of CHF1,188m

We value Basilea at CHF1,188m or CHF110/share, which is based on a risk-adjusted NPV (rNPV) analysis, including Cresemba and Zevtera, in addition to indicative valuations for the earlier-stage pipeline (BAL101553 and BAL3833). Our valuation suggests the current share price is more than underpinned by the potential for both Cresemba and Zevtera where approvals have already been granted, with the market ascribing seemingly limited value to Zevtera in the US and the earlier-stage oncology pipeline. Our revised valuation reflects in the main changes to our Cresemba assumptions; we have increased our non-US peak sales expectations. Basilea will benefit from royalties and milestones on Cresemba sales. Tweaks to our Zevtera numbers include a small cut to our EU expectations but have increased ROW following the licencing deal with CR Gosun).

Sensitivities: Commercialisation is key

The key sensitivities for Basilea, in our view, relate to successful commercialisation (largely through respective partners) of both Cresemba and Zevtera (where approved), progress with the Phase III US Zevtera trials and crystallising value from the earlier-stage pipeline. Both Cresemba and Zevtera are targeting significant markets; however, we believe there are two main challenges to uptake: (1) related to Zevtera antimicrobial stewardship (described later in this report); and (2) the availability of cheaper generic treatments. Success for both assets (Cresemba US and EU, Zevtera US) is largely in the hands of existing, or prospective, partners where we have limited visibility.

Financials: Sufficient cash beyond profitability

We believe that Basilea's gross cash (including financial investments) at 30 June 2017 of CHF253.1m (this excludes the CHF70m upfront from Pfizer received in July 2017) should be sufficient to fund operations beyond 2019 even excluding future potential deals for either Zevtera in the US or the earlier-stage assets. Profitability will be driven by royalties and milestones on sales of Cresemba worldwide and Zevtera in Europe/ROW. We assume an uptick in R&D spend in 2018/19 owing to Basilea's potential contribution towards the Zevtera US Phase III clinical development; we assume a related R&D expense of around CHF40m for both trials (BARDA funding amounts to $108m for three trials over the 4.5-year period from April 2016). However, our expectations for sales and marketing expenses have been lowered reflecting the partnering deal for Cresemba with Pfizer; and the distribution agreement with Cardiome for Zevtera.

Full steam ahead

2017 has been a successful year to date. Basilea has announced multiple licensing deals for its commercially available products Cresemba and Zevtera. This includes several distribution deals; Zevtera (Cardiome in Europe ex Nordics and Israel), Zevtera and Cresemba (Avir Pharma in Canada). Additionally, Basilea has signed two notable licence agreements: 1) Zevtera in China with CR Gosun (CHF 3m execution payment plus up to CHF 145m in additional payments in regulatory and commercial milestones); and 2) the major licensing deal with Pfizer for Cresemba in Europe (ex-Nordics), Russia, Turkey and Israel. Pfizer is the partner of choice given its longstanding expertise in this market. With both Zevtera and Cresemba's commercialisation strategy largely in the hands of partners, Basilea can turn its focus to its next pillars of growth: Zevtera's Phase III US clinical trial programme initiation (plus potential partnering) and investing in the early to mid-stage products (BAL101553 and BAL3833), which are targeting cancer resistance. Importantly the deal with Pfizer not only validates Cresemba's potential but increases the financial fire power to bolster Basilea's existing, innovative R&D pipeline offering.

Cresemba a done deal

Basilea has been commercialising both Cresemba and Zevtera alone in key European markets through a fee-for-service contract salesforce via an agreement with Quintiles. In April 2015 partner Astellas launched Cresemba in the US market, where the product is garnering momentum; Astellas has guided $77m in sales for the financial year 1 April 2017 to 31 March 2018. The licensing deal with Pfizer means that in key worldwide markets partners are responsible for Cresemba commercialisation, with Basilea eligible for royalties on sales plus milestones. Basilea has other licence agreements in place outside the US and Europe for Cresemba (Exhibit 1); however, it is not currently approved outside the US and EU. We have increased our Cresemba peak sales forecasts as we believe Pfizer's marketing might and expertise will drive higher penetration in its in-licensed territories.

US Zevtera opportunity rests on Phase III data

Zevtera is approved in Europe but uptake in this market remains slow as sales of antibiotics take time to build post launch due to the requirement for regional reimbursement across Europe and the need to be added to individual hospital formularies. Zevtera is currently not approved in the US where we believe the bulk of its sales opportunity resides; in terms of value. Zevtera's Phase III US clinical programme will be initiated in Q417 (ABSSSI study) and H118 (bacteraemia study), following the FDA agreement on the Special Protocol Assessments for the two Phase III clinical studies in *Staphylococcus aureus* bacteraemia and acute bacterial skin and skin structure infections. Critically, the funding for these trials plus trial in community acquired pneumonia is in place with up to $108m from BARDA and Basilea funding the deficit. We forecast a launch within the US in 2022.

Next up: Focus on profitability and pipeline growth

We believe Basilea can achieve sustainable profitability from 2020. Lower operating costs from 2018 offset by increased R&D (although BARDA remains a major source of funding for the US Phase III Zevtera development programme) with the backdrop of significant royalty and milestone income streams will be transformative for the bottom line. This coupled with a strong balance sheet should enhance Basilea's ability to bolster the pipeline through in-licensing of early stage products to complement its existing early to mid-stage cancer resistance focused R&D portfolio. Basilea's oncology clinical pipeline contains BAL101553 (water soluble pro-drug of BAL27862) and BAL3833 for drug-resistant cancers. Phase I data of the Phase I//IIa trial is expected in 2018.

Pfizer deal is a game changer for Cresemba

Cresemba (isavuconazole) is a broad-spectrum antifungal for the treatment of severe, life-threatening fungal infections. Invasive fungal infections are a major cause of morbidity and mortality in immunocompromised patients (eg cancer, transplant patients). Mortality rates for invasive fungal infections vary by pathogen: 23-40% for *Candida*, 34-58% for *Aspergillus* and 40-80% for mucorales (Kullberg/Arendrup NEJM 2015); Baddley, *Clinical Infectious Diseases* 2010; Roden, *Clinical Infectious Diseases* 2005; Greenberg, *Current Opinion in Infectious Diseases* 2004). There remains a need for new agents to treat invasive fungal infections: drugs that have a broad-spectrum activity against moulds, including emerging moulds such as mucorales. Widely available fungal treatments such as Pfizer's Vfend (voriconazole) and Gilead's AmBisome (liposomal Amphotericin B) have limitations to the spectrum of activity and toxicity profile, while AmBisome is only available in intravenous formulation. Cresemba is the only available 'azole' that has mucormycosis on the label; voriconazole and posaconazole are not approved for the treatment of invasive mucormycosis.

Cresemba was approved in the US in March 2015 for the treatment of both invasive aspergillosis and invasive mucormycosis and has been launched by US partner Astellas Pharma. Cresemba was approved in Europe in October 2015 and Basilea has been commercialising Cresemba itself in the major European markets (launched in the UK, Germany, Italy, Austria and France so far). Partner Unimedic has begun commercialisation in the Nordic regions. Pfizer will be responsible for future commercialisation in Europe (ex Nordics) and we expect further European launches during 2017/18 (pricing and reimbursement need to be agreed in each country, which takes time). Cresemba has been awarded orphan drug status in both the US and Europe. In the US it has exclusivity through 2027 based on seven years under orphan drug status and an additional five years of exclusivity under qualified infectious disease product (QIDP) designation. In Europe exclusivity is based on 10 years under orphan drug designation plus a further two years' exclusivity could be granted on the basis of completion of the paediatric investigation plan (PIP).

Basilea made significant progress in partnering deals during 2016 and 2017. Its existing Cresemba partnerships now cover more than 80 countries internationally. Exhibit 1 highlights the existing partnerships for both Cresemba and Zevtera; we note in many instances partners have chosen to in-license both products given the significant overlap in the physician prescribing base.

Exhibit 1: Cresemba and Zevtera partners/distribution agreements

Product	Partner/Distributor *	Territory	Comments
Cresemba	Astellas	US	CHF117m upfront and regulatory milestones received with up to CHF290m of sales milestones. Tiered royalty starting in the mid-teens and ramping up to mid-20s on sales
Cresemba	Pfizer	Over 40 countries in Europe (excluding Nordics), Russia, Turkey and Israel	CHF70m upfront and up to US$427m sales and regulatory milestones plus mid-teen on sales royalties
Zevtera	Cardiome	Europe (excluding Nordics) and Israel	Upfront CHF 5m and regulatory and commercial milestone payments. Participate in sales through a transfer price.
Zevtera	CR Gosun	China, Hong Kong and Macau	CHF3m execution payment and up to CHF145m additional payments upon achievement of pre-specified regulatory and commercial milestones.
Cresemba and Zevtera	Unimedic Pharma *	Nordic countries including Sweden, Denmark, Norway, and Finland	Upfront and sales milestone payments. Participate in sales through a transfer price.
Cresemba and Zevtera	Grupo Biotoscana (GBT)*	19 countries in Latin America including Brazil, Mexico, Argentina and Colombia	CHF11m upfront plus milestone payments. Participate in sales through a transfer price.
Cresemba	Asahi Kasei Pharma (AKP)	Japan	CHF7m upfront and up to CHF60m regulatory and commercial milestone payments plus double-digit tiered royalties
Cresemba and Zevtera	Avir Pharma *	Canada	Upfront and sales milestone payments. Participate in sales through a transfer price.
Cresemba and Zevtera	HIKMA*	MENA region	Financial terms not disclosed. Participate in sales through a transfer price.

Source: Edison Investment Research, Basilea Pharmaceutica. Note: *Distribution agreements where Basilea supply product as a transfer price.

US Cresemba sales steadily growing

In the US, partner Astellas has reported sales of $34m for January to June 2017 and has guided $77m in sales for the financial year 1 April 2017 to 31 March 2018. Basilea is entitled to a tiered royalty on Cresemba sales starting in the mid-teens and ramping up to mid-20s with up to CHF290m of sales milestones. Our peak sales forecast for Cresemba includes $250m for the US opportunity; we believe Cresemba could reach our expectations at its current sales trajectory.

Cresemba Pfizer tie-up strategic and commercial sense

While the Pfizer licensing deal for Cresemba came as a surprise, we view the deal to be highly value accretive in maximising shareholder value. Basilea has been commercialising Cresemba alone in key European markets (including the UK, Germany, Italy, France and Austria) through a fee-for-service contract salesforce via an agreement with Quintiles. This agreement provided dedicated sales representatives who are trained by Basilea, in addition to market access and administrative support. As such, the European opportunity represented a smaller part (c 25%) of our original $600m global peak sales forecasts.

The licensing deal with Pfizer is a game changer for Cresemba and makes strategic and commercial sense. Pfizer has the expertise to manufacture and commercialise Cresemba rapidly throughout its in-licensed territories using its established marketing infrastructure during a period where its own anti-fungal Vfend has lost patent protection. Under the terms of the deal, Basilea has received an upfront payment of CHF70m and will be eligible to receive up to $427m upon achievement of pre-specified regulatory and sales milestones. Basilea will also receive royalties in the mid-teen range on Pfizer's sales in the territories mentioned. Basilea will no longer benefit from booking Cresemba sales in Pfizer partnered markets, however it could benefit from substantial milestone payments and royalties on sales. Furthermore, we expect a decrease in the commercialisation costs for Cresemba in Europe currently borne by Basilea. In other partnered territories (Canada, Nordics, Latin America and the MENA region) Cresemba sales will be booked at the product transfer price to the relevant partner plus prespecified commercial milestones.

Pfizer's own anti-fungal Vfend and competitor AmBisome (Gilead) are in the process of losing patent protection in key markets. Pfizer reported Vfend global sales of $682m in 2015, down from the 2010 peak of $825m following the availability of oral generics in 2011, in addition to an iv generic in 2012 in the US market; and full genericisation in Japan (January 2016) and the major European markets (July 2016). Gilead's AmBisome (amphotericin B), which is used to treat invasive mucormycosis, among other fungal infections, had global sales of $378m in 2016 ($373m in 2015); the US patent expired in 2016. IMS Health reported total voriconazole (branded Vfend plus available generic) sales of $902m in 2013, and a combined market of $2.7bn in 2016 based on sales of approved leading antifungals (the bulk of this relates to Vfend brand and generics and Merck's Noxafil [posaconazole], which reported $581m in global sales in 2016).

Cresemba is non-inferior to Vfend on efficacy

Cresemba was approved on the basis of two Phase III trials in mould infections: SECURE, a 516-patient trial comparing Cresemba to Vfend in patients with invasive aspergillosis, in addition to the VITAL open-label trial in 156 patients, including rare moulds and patients with invasive aspergillosis with renal impairment. Cresemba was found to be non-inferior to Vfend in terms of efficacy (as measured by all-cause mortality at day 42), with a more favourable safety profile, reporting statistically fewer drug-related adverse events; in particular, there were significantly fewer liver, eye and skin disorders (all known effects of Vfend). Importantly, according to the Cresemba product label, there is no need for dose modification in mild, moderate or severe renal disease nor in mild or moderate liver impairment. This directly sets Cresemba apart from Vfend. The main data are summarised in Exhibit 2.

Exhibit 2: Main data from the Phase III SECURE study

	Cresemba	Vfend	Statistical outcome	
Intent-to-treat population (ITT)			Difference	95% CI
Number of patients	258	258		
All-cause mortality (day 42); n (%)	48 (18.6%)	52 (20.2%)	-1.0%	-8.0% to +5.9%
In patients with proven/probable invasive fungal disease (mITT, modified ITT)			Difference	95% CI
Number of patients	123	108		
All-cause mortality (day 42); n (%)	23 (18.7%)	24 (22.2%)	-2.7%	-13.6% to +8.2%
Safety				
Drug-related adverse events	42.4%	59.8%	p=ss	
Hepatobiliary disorders	8.9%	16.2%	p=0.016	
Skin disorders	33.5%	42.5%	p=0.037	
Eye disorders	15.2%	26.6%	p=0.002	

Source: US Cresemba label; Maertens et al., *The Lancet*, 2016. Note: CI=confidence interval; ss = statistically significant.

Cresemba is differentiated on safety

Cresemba offers safety advantages over current treatments. The intravenous formulation of Vfend is not recommended to treat patients with moderate to severe renal impairment and the dose has to be lowered in patients with liver impairment. It also has a number of drug interactions and is associated with visual disturbances. Vfend is not approved to treat invasive mucormycosis, where until the Cresemba approval, treatment was limited to amphotericin B, and its newer liposomal formulation (Astellas/Gilead) AmBisome (only available in intravenous formulation), which is associated with infusion-related events and nephrotoxicity. Cresemba has a broad spectrum of activity including mucormycosis; it is well suited to empiric use when the exact underlying microbial cause of infection is unknown. This is particularly important with severe, life-threatening invasive fungal infections, where timely initiation of treatment is important. Diagnosing the causative pathogen is not always straightforward; for example, mucormycosis can be difficult to differentiate from other filamentous fungi such as aspergillosis. These features should therefore differentiate Cresemba from the competition, potentially providing physicians with an option that overcomes the limitations of current therapy.

Upgrade peak sales to $0.8bn from $0.6bn

We believe Pfizer is in an optimal position to capitalise on Cresemba's unique profile among antifungals (broad-spectrum activity plus lower toxicity). We note that Basilea estimates (source: QuintileIMS SMART MIDAS May 2017) that the top 5 European countries represent 32% of the antifungal market and the US represents 24%. We believe Pfizer is well positioned to target a wider range of hospitals and patients than Basilea was able to, and as such have increased our penetration rates and resulting peak sales forecasts. We now forecast a peak penetration of 45% in the Pfizer markets. The result is an increase in our peak sales forecasts to $0.8bn from $0.6bn. Additionally, while uncertainty remains on the accounting for the Cresemba upfront payment(CHF70m), we have deferred it as relative to the Pfizer sales over an estimated period of four to five years.

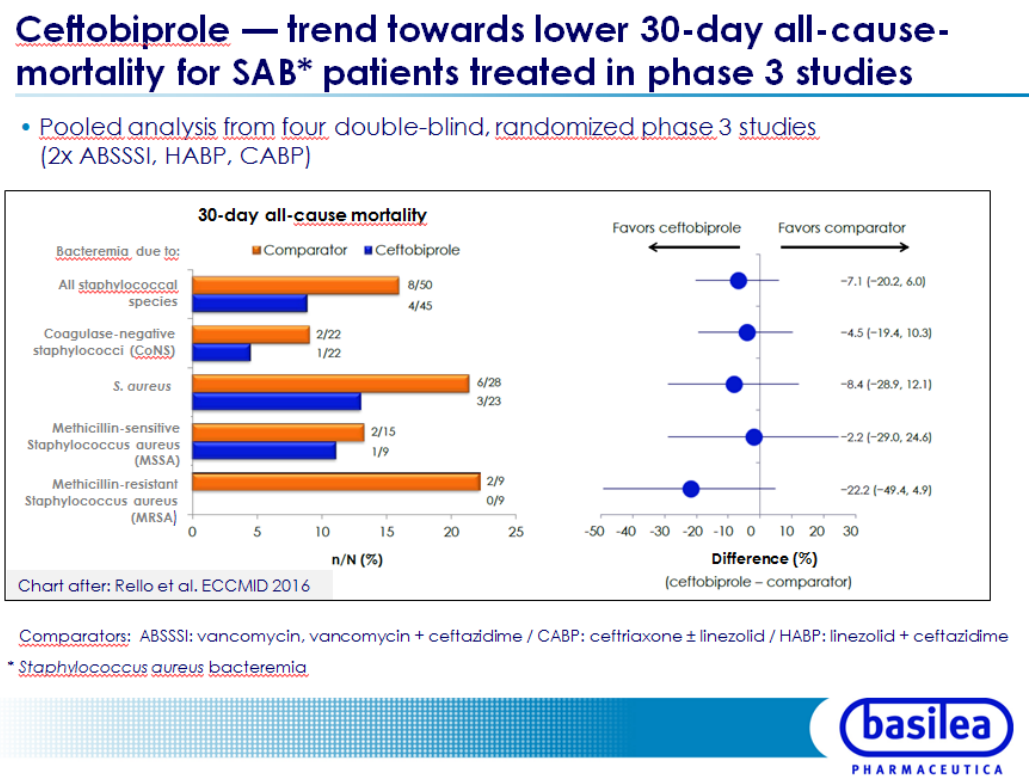
We believe the main competition for Cresemba will be from increasing genericisation of the current invasive fungal infections therapy market, as Vfend is becoming fully genericised. However, despite the availability in the US of oral generics since 2011 (in addition to the first I.V. generic in 2012), US sales have only been marginally affected to date, declining 8% from the 2010 peak. Vfend illustrates those products with improved profiles such as safety can gain and maintain a strong foothold in markets, despite the then availability of generic fluconazole. Furthermore, despite the availability of generics, the antifungal market grew at a 17% CAGR from 2004-15 in volumes, driven by growth in underlying patient populations and the replacement of (cheaper) legacy antifungals with newer antifungals.

Zevtera fortunes reside in US Phase III outcome

Zevtera/Mabelio (ceftobiprole) is a broad spectrum antibiotic for the treatment of Gram-positive, including MRSA (methicillin-resistant *Staphylococcus aureus*) infections, that are resistant to a number of existing antibiotics, and Gram negative bacterial infections, including *Pseudomonas*. The recently commissioned Review on Antimicrobial Resistance estimates that around 700,000 people currently die each year globally from drug-resistant infections, with this projected to increase to 10 million by 2050. Importantly, the WHO has reported that antibiotic resistance is a serious, worldwide threat to public health, and states that "people with MRSA are estimated to be 64% more likely to die than people with a non-resistant form of the infection".

Zevtera was approved in Europe for hospital-acquired and hospitalised community-acquired pneumonia (excluding ventilator-associated pneumonia) towards the end of 2013. Basilea is commercialising Zevtera alone in core European markets through a fee-for-service contract salesforce in place through Quintiles. In the US, further clinical studies are needed to secure approval; the Phase III Special Protocol Assessments (SPAs) have been agreed with the FDA and both the *Staphylococcus aureus* bacteraemia (bloodstream infections) and acute bacterial skin and skin structure infections trials will start in H118 and Q417, respectively. The cross-supportive studies are part of the clinical Phase III programme aiming at regulatory approval of ceftobiprole in the United States. The bacteraemia studies could be an important source of differentiation; a pooled analysis from four double-blind, randomised Phase III studies (two in acute bacterial skin and skin structure infections, one in hospital acquired bacterial pneumonia and one in community acquired bacterial pneumonia) showed a trend toward lower 30-day all-cause mortality in patients with *Staphylococcus aureus* bacteraemia (Exhibit 3).

Exhibit 3: Ceftobiprole: Bacteraemia pooled data analysis



Source: Basilea Pharmaceutica

A vast market for hospital antibiotics

There are a number of branded hospital antibiotics for use in severe bacterial infections. These include Pfizer's Zyvox and Merck's Cubicin. Zyvox, which is approved for Hospital Acquired Pneumonia (HAP), Community acquired Pneumonia (CAP) and complicated skin and skin structure infections (cSSSI), including diabetic foot infections, reported 2014 sales of $1.35bn (in the year before its patent expiry, $883m in 2015, $421m in 2016). Cubicin (originally sold by Cubist in the US, which was acquired by Merck in early 2015) last reported US sales of $908m (by Cubist in 2013). Cubicin is approved for cSSSI and bloodstream infections including right-sided endocarditis, but is not approved for pneumonia, having failed in clinical trials. Cubicin had a 14% share of the US market for iv antibiotics to treat serious gram-positive infections, based on days of therapy (according to Cubist's 2013 annual SEC filing). Other branded hospital antibiotics include, but are not limited to, Tygacil (Wyeth/Pfizer), Vibativ (Theravance) and Teflaro (Forest/Actavis/Allergan). In addition, vancomycin is available generically and has around a 70% share of the US market (based on days of therapy).

US peak sales estimate of $317m

Given the size of the worldwide anti-MRSA antibiotic market ($3.8bn in 2015), even a small market share could make significant peak sales possible. Basilea estimates that in terms of value, the US represents about 70-80% of the total global market for newer, branded hospital antibiotics. However, despite Cubicin's success in the US (which demonstrates the willingness of US physicians to embrace new antibiotics despite the availability of cheaper generic options), in Europe uptake of new antibiotics has been slower. We believe this reflects a general caution in Europe around use of new antibiotics unless absolutely necessary, to prevent future antimicrobial resistance (also referred to as "antimicrobial stewardship"). Zevtera uptake in Europe remains slow, and as a result we have lowered our EU peak sales expectations to $60m in 2023 (for the currently approved indications). We anticipate that peak sales in the different regions will likely not be reached at the same time given launches in China and the US are later than Europe and other RoW countries. We have increased our ROW expectations slightly following the licence deal with CR Gosun in China; we now forecast ROW peak sales of $168m in 2025, and US peak sales of $317m in 2027, which is predicated on securing a partner. Phase III data from the US trials, if positive, could be used to expand Zevtera's EU/RoW label to include *Staphylococcus aureus* bacteraemia and acute skin and skin structure infection indications. Our current peak sales estimates for EU/RoW do not include these indications, hence this could provide upside to our financial forecasts.

Strategy for the US market

Basilea has reached an agreement with the FDA on Special Protocol Assessments for the two Phase III clinical studies in *Staphylococcus aureus* bacteraemia (and acute bacterial skin) and skin structure infections. These two cross-supportive studies will initiate in H217 (we expect them to take 18-24 months in skin infections and 3 years in bacteraemia); both trials will be needed to support a US NDA submission and post marketing label extensions in countries where Zevtera is commercially available. A US 2022 launch date could be feasible, with an initial focus of bacteraemia and acute bacterial skin and skin structure infections. *Staphylococcus aureus* bacteraemia (presence of bacteria in the blood) is an indication where few antibiotics are currently approved and which the FDA considers an area of unmet need; therefore, the bacteraemia study could further differentiate Zevtera from available cephalosporins.

In March 2016, the Biomedical Advanced Research and Development Authority (BARDA), a division of the US Department of Health and Human Services Office of the Assistant Secretary for Preparedness and Response, entered into a contract with Basilea for the Phase III development of

Zevtera for the US market. The SPA agreement has triggered the commitment of €54.8m in reimbursements from BARDA for two additional options under its existing contract. The $58.4m from BARDA adds to the $20m available for reimbursement triggered in 2016; the total value of the contract now stands at $108m (from $100m) over a period of 4.5 years if certain pre-defined milestones are achieved.

Importantly, the BARDA contract provides a path to create further value by enabling non-dilutive funding for the programme before seeking a commercialisation partner for the US market. Additionally, results from the US clinical trial programme could be used to support supplementary indications in Europe and other territories, leveraging Zevtera's potential use further.

Zevtera has been awarded QIDP designation in the US for both pneumonia and skin infections, which should provide at least 10 years of market exclusivity (including an additional five years of exclusivity in the US on top of standard data exclusivity of five years for a new chemical entity) from the date of approval.

Oncology pipeline assets data expected in 2018

To complement the primary focus on anti-infectives, Basilea also has an early-stage pipeline focused on oncology products that target resistance to current traditional chemotherapies. Basilea's oncology clinical pipeline contains BAL101553 and BAL3833 for drug-resistant cancers. There could be synergies between the anti-infectives and oncology pipelines as a large number of invasive fungal and bacterial infections develop in cancer patients that are immunocompromised due to treatment with aggressive chemotherapies. The readout of the dose-escalation parts of the Phase I/IIa and Phase I studies evaluating BAL101553 and BAL3833 respectively in patients with solid tumours are duein 2018.

BAL101553 novel tumour checkpoint controller

BAL101553 is a highly water-soluble prodrug of BAL27862, which induces tumour cell death through activation of a checkpoint important for tumour cell division. Once administered into the body, BAL101553 is converted to the active form, BAL27862. It targets microtubules, but with a binding site and mechanism of action distinct from that of currently approved microtubule-targeting agents (MTA; such as Taxol, Taxotere, Abraxane, Jevtana and the Vinca alkaloids). BAL101553 has shown anticancer activity in a number of treatment-resistant tumour models, including tumours resistant to standard MTAs, as well as other therapeutic approaches including radiotherapy. Furthermore, BAL27862 has been observed to affect tumour blood supply in preclinical models and has also been shown to activate a checkpoint involved in preventing cell proliferation; therefore it could have utility across multiple tumour types. BAL27862 has also been shown to have penetration into the brain; thereby supporting the rational to extend the ongoing phase I/IIa study to include patients with glioblastoma. Basilea announced earlier this year that it has entered into a clinical agreement with the Adult Brain Tumour Consortium (ABTC) to test BAL101553 in newly-diagnosed glioblastoma patients. The consortium will conduct a Phase I trial to study the safety and tolerability of the drug in combination with radiotherapy. Patients will be selected who are unlikely to respond to chemotherapy as determined by the detection of an unmethylated MGMT promoter, a key biomarker for glioblastoma patients. Finally, an ongoing Phase I/IIa study is studying the effect that weekly 48 hour continuous infusion of BAL101553 has on solid tumours.

BAL3833 for BRAF resistance and other refractory solid tumours

BAL3833 is a panRAF/SRC kinase inhibitor kinase inhibitor in Phase I development in advanced solid tumours. It was in-licensed by Basilea in April 2015 under an agreement with a consortium of organisations including The Institute of Cancer Research, London, Cancer Research Technology,

the Wellcome Trust, and The University of Manchester. It inhibits both BRAF and CRAF, part of the RAF family of kinases, and inhibits the SRC kinase family, which are involved in cell growth. BRAF mutations are found in certain cancers, most notably melanoma, with BRAF inhibitors such as Zelboraf approved to treat melanoma patients with a BRAF mutation. Preclinical data suggest that BAL3833 has activity in models resistant to current BRAF inhibitors.

Preclinical data also suggest that BAL3833 has activity in KRAS-driven cancer models, suggesting it could have clinical utility in major tumour types beyond BRAF-driven melanoma. It is currently being tested in a Phase I dose escalation study; patient enrolment is ongoing and the study aims to determine the maximum tolerated. Completion of patient recruitment is expected within the next six to nine months.

Sensitivities

Basilea is subject to the usual biotech and drug development risks, including clinical development delays or failures, regulatory risks, competitor successes, partnering setbacks, and financing and commercial risks. The key sensitivities for Basilea relate to successful commercialisation of both Cresemba and Zevtera in the respective approved territories, progress of the Zevtera Phase III programme in the US and crystallising value from the earlier-stage pipeline.

Cresemba is already approved in most of the key territories worldwide; hence the focus is on successful commercialisation and is largely in the hands of existing or prospective partners. We believe Astellas is well placed to deliver on Cresemba US sales, with Cresemba one of its key products and an already existing commercial presence in anti-infectives. In the major European markets, Basilea has been commercialising the product through a contract salesforce via Quintiles, with dedicated sales reps and Basilea responsible for training. With the out-licensing of European market (plus Russia, Turkey and Israel) to Pfizer, the risks of commercialisation relate to Pfizer's strategy in these territories. In the US, Basilea is seeking a US partnership for Zevtera, but we have limited visibility on the timing and terms of any deal.

In general, we believe there are two main challenges to Zevtera and Cresemba uptake: (1) related to Zevtera antimicrobial stewardship, particularly in Europe where novel anti-infectives are often used with caution to try and prevent the emergence of new resistant strains; and (2) the availability of cheap generic options, particularly in an era of pricing pressures and restricted budgets.

For the earlier-stage pipeline, both clinical development and partnering risks remain. We expect Basilea to develop each oncology asset (BAL101553 and BAL3833) through to Phase II with initial proof-of-concept data before partnering. However, we have limited visibility beyond that on the terms and timing of any potential deal(s).

Valuation

Our updated Basilea valuation is CHF1,188m (from CHF1,048m previously), primarily as a result of adjustments to our Cresemba assumptions (post the PFE deal) and Zevtera forecasts. Additionally, we roll forward our DCF and adjust for a lower net cash position at end June 2017 (CHF57.3m vs CHF93.6m at end December 2016). We highlight that the end June 2017 net cash does not include the CHF70m received from Pfizer in July after completion of the transaction. Our valuation is based on an NPV analysis, which includes the main portfolio of products and net cash. Cresemba, based on $0.8bn peak sales, is worth CHF913.5m. We also include Zevtera in Europe, in addition to risk-adjusted contributions for the US opportunity and the earlier-stage pipeline. The breakdown of our valuation is shown in Exhibit 4.

Exhibit 4: Basilea rNPV valuation

Product	Indication	Launch	Peak sales ($m)	Value (CHFm)	Probability	rNPV (CHFm)	NPV/share (CHF/share)
Cresemba (isavuconazole)	Severe fungal infections	2015 (US); 2016 (EU); 2018 (ROW) 2020 Japan	824	948.5	75 -100%*	913.5	84.6
Zeftera/Mabelio (ceftobiprole)	Severe bacterial infections	2015 (EU); 2018 (RoW ex China); 2019 (US); China 2022	US 317, EU 60, ROW 168	235.3	100%**	193.4	17.9
BAL101553	Tumour resistance	2023	500	126.3	20%	18.6	1.7
BAL3833	Tumour resistance	2024	500	92.3	15%	5.4	0.5
Net Cash/(Debt)				57.3	100%	57.3	5.3
Valuation				1,459.6		1,188.2	110.0

Source: Edison Investment Research. Note: *100% probability for US and EU, 75% for ROW and Japan; **100% probability for EU, 75% probability for China, ROW and US.

For Zevtera in Europe, revenue forecasts now include product sales at transfer prices received from partner Cardiome. For Zevtera in the US, Basilea is seeking a commercial partnership; our valuation includes an assumed deal with a total value of CHF100m (a base case assumption, which could prove conservative) and a 20% royalty on sales. Our valuation includes an assumed 30% contribution by Basilea towards the cost of Phase III development ($90-120m total cost for two Phase III trials). With efficacy already demonstrated in previous trials for skin and lung infections, we believe the biggest risk to Zevtera in the US is around partnering, where we have limited visibility on the likelihood of securing any deal. Hence we apply a 75% probability of success; however, we note the data for Zevtera in Europe has been better in some indications versus others. For the rest of world (ex-US and ex-EU), we assume a rising transfer pricing on sales of 35%-50% for both Cresemba and Zevtera; note the bulk of our sales for each is in the US and Europe.

In the US, Cresemba is partnered with Astellas and our valuation includes tiered future royalties on sales starting from the mid-teens ramping up to mid-20s. Basilea is also entitled to up to CHF290m of sales-related milestones, some of which are included in our valuation. In the EU we have increased out sales assumptions based on Pfizer's ability to reach more patients as a result of a larger salesforce and existing infrastructure. We now assume a 15% royalty and on peak sales in the EU of CHF400m Basilea would collect CHF60m. Of the $427m in regulatory and sales milestones which Basilea is eligible to receive from Pfizer we have included CHF400m in our model in the 2017 to 2025 timeframe.

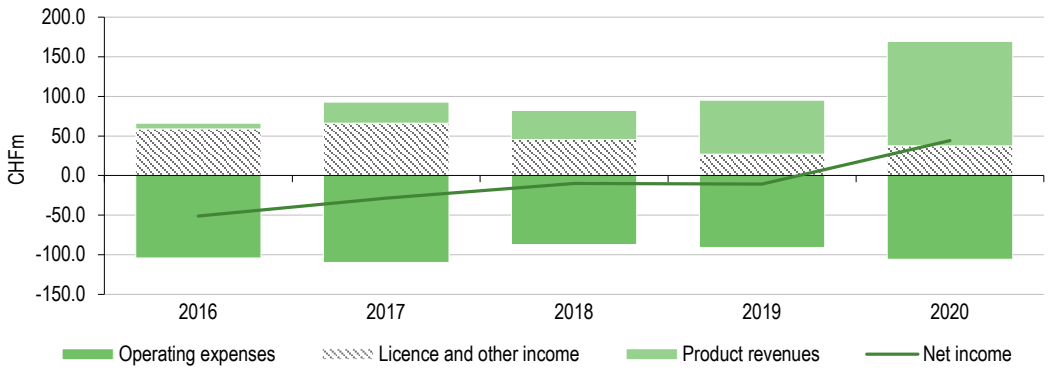
We also include indicative valuations for BAL101553 and BAL3833 and for simplicity we assume both will be partnered post completion of Phase II trials, in exchange for a royalty on sales (starting at 15% for both, given we have assumed partnering once proof-of-concept data become available). Assessing the potential for each product is challenging in the absence of proof-of-concept data and without knowing the indications that will be pursued in the future. For both we include peak sales of $500m; this will ultimately be determined by which future oncology indications are developed, but we believe it is a reasonable base case assumption for an oncology asset that could have use in a variety of drug-resistant solid tumours.

Financials: FY17 guidance uplift

Basilea reported H117 product sales of CHF9.8m on sales of anti-microbial agents Cresemba and Zevtera/Mabelio in Europe and royalties on US Cresemba sales of CHF5.2m. Total revenues increased 56% in H117 (reported at CHF46.2m) vs H116 (reported at CHF29.7m). The company reported CHF5.2m in royalties on US Cresemba sales. Astellas has guided $77m in sales for the financial year 1 April 2017 to 31 March 2018. Given Cresemba's strong sales momentum in the US and the announcement of the regulatory approval of the Pfizer deal for Europe (ex Nordics), Russia, Turkey and Israel, we have significantly increased our peak sales forecasts for Cresemba

to $0.8bn from $0.6bn. While the accounting treatment for the Pfizer license fee is unknown at this time, we have assumed the CHF70m is deferred over five years from the date of the announcement as relative to the Pfizer sales over the period while the cash impacts 2017 alone.

Exhibit 5: Forecasted opex, licence income, product revenues and net income



Source: Edison Investment Research, Basilea Pharmaceutica

Costs and operating expenses were higher than the previous period, at CHF63.9m (H117) vs CHF54.6m (H116). The increase was driven mainly by an increase in SG&A costs to CHF33.9m (CHF26.8m), with smaller increases in R&D (H117: CHF26.4m; H116: CHF24.8m) and cost of products sold (HY17: CHF3.5m, HY16: CHF3.0m). We note that our cost of sales assumptions relate purely to cost of goods sold; this differs from Basilea's reported cost of sales, which includes manufacturing, capacity reservation, shipping and handling costs (Edison accounts for these costs in SG&A). Thus, during the launch period, cost of sales as reported by Basilea is higher than our forecast cost of sales. We forecast 2017 gross R&D spend of CHF63m and CHF79m in 2018. Net R&D (taking into consideration of BARDA reimbursement for the two US Zevtera trials) for 2017 is CHF58m in both 2017 and 2018. We assume an uptick in R&D spend in 2018/19 owing to Basilea's potential contribution towards the Zevtera US Phase III clinical development; we assume a related R&D expense of around CHF40m for both trials (BARDA funding amounts to $108m for three trials over the 4.5-year period from April 2016). Future R&D spend will also depend on partnering activities for the earlier-stage assets, although we do not expect any deals for either BAL101553 or BAL3833 until Phase II proof-of-concept data become available.

Overall, we now forecast total operating expenses of CHF110.2m in 2017 from CHF104.5m in 2016. We forecast an operating loss of CHF23.3m for FY17, which is in line with the company guidance of a CHF2m average operating loss per month. For 2018 we expect the reduction in European Cresemba and Zevtera related SG&A (due to the PFE licensing deal and the Cardiome distribution deal respectively) to lower operating expenses to CHF87.7m for the year. However, some sales and marketing costs will remain to support the core commercialisation strategy of Zevtera in Europe.

Exhibit 6: Our revised forecasts are in line with 2017 revised company guidance*

2017	Outlook	Edison estimates
Operating expenses	CHF9-10m/month	CHF9.2/month
Operating loss	CHF2m/per month	CHF2.0/month
Product sales	CHF13m	CHF10.9m
Partner royalties	CHF15m	CHF15.8m

Source: Basilea Pharmaceutica, Edison Investment Research. Note: *Basilea's guidance was last updated in August 2017.

Cash could be sufficient beyond 2019

Basilea reported cash and equivalents, including liquid assets, of CHF253.1m at end June 2017 (does not include the July CHF70m upfront from Pfizer) compared to CHF289.0m at year-end December 2016. This includes proceeds from the convertible bond, which we record on the balance sheet as long-term debt at CHF195m. The convertible bond is due in 2022 and has a conversion price of CHF126 (based on a 30% premium to the volume weighted average share price on 9 December 2015, which was CHF97). The coupon is 2.75%, or CHF5.5m/year, which is paid semi-annually in arrears. Our financial model suggests current cash should be sufficient to fund operations beyond 2019, even in the absence of any milestone payments.

Exhibit 7: Financial summary

CHF'000s	2015	2016	2017e	2018e	2019e
December	US GAAP	US GAAP	US GAAP	US GAAP	US GAAP
PROFIT & LOSS					
Revenue	52,825	65,984	92,404	82,209	95,173
Cost of Sales	0	(5,347)	(5,500)	(6,886)	(9,695)
Gross Profit	52,825	60,637	86,904	75,323	85,478
Research and development (gross)	(60,075)	(48,449)	(63,000)	(79,000)	(86,000)
EBITDA	(58,885)	(41,570)	(20,872)	(9,824)	(2,925)
Operating Profit (before amort. and except.)	(61,285)	(43,789)	(23,199)	(12,282)	(5,516)
Intangible Amortisation	(200)	(100)	(100)	(123)	(9)
Exceptionals	0	0	0	0	0
Other	0	0	0	0	0
Operating Profit	(61,485)	(43,889)	(23,299)	(12,405)	(5,525)
Net Interest	(35)	(7,065)	(5,375)	(5,375)	(5,375)
Profit Before Tax (norm)	(61,320)	(50,854)	(28,574)	(17,657)	(10,892)
Profit Before Tax (reported)	(61,520)	(50,954)	(28,674)	(17,780)	(10,900)
Tax	(83)	(333)	(26)	(26)	(26)
Profit After Tax (norm)	(61,403)	(51,187)	(28,601)	(17,683)	(10,918)
Profit After Tax (reported)	(61,603)	(51,287)	(28,701)	(17,806)	(10,926)
Average Number of Shares Outstanding (m) excluding treasury shares	10.1	10.1	10.1	10.1	10.1
EPS - normalised (c)	(607.22)	(505.74)	(282.58)	(174.72)	(107.87)
EPS - normalised fully diluted (CHFc)	(607.22)	(505.74)	(282.58)	(174.72)	(107.87)
EPS - (reported) (CHFc)	(6.09)	(5.07)	(2.84)	(1.76)	(1.08)
Dividend per share (CHFc)	0.0	0.0	0.0	0.0	0.0
Gross Margin (%)	100.0	91.9	94.0	91.6	89.8
EBITDA Margin (%)	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
BALANCE SHEET					
Fixed Assets	13,870	59,264	59,569	59,454	59,710
Intangible Assets	346	232	132	9	0
Tangible Assets	10,724	8,878	9,283	9,292	9,556
Investments	2,800	50,154	50,154	50,154	50,154
Current Assets	384,865	268,494	255,014	207,503	177,410
Stocks	9,579	14,931	10,710	9,433	7,969
Debtors	1,545	2,492	2,532	2,252	2,607
Cash	364,688	239,030	229,732	183,777	154,793
Other	9,053	12,041	12,041	12,041	12,041
Current Liabilities	(68,836)	(72,914)	(64,601)	(45,781)	(58,260)
Creditors	(68,836)	(72,914)	(64,601)	(45,781)	(58,260)
Short term borrowings	0	0	0	0	0
Long Term Liabilities	(315,043)	(289,844)	(308,723)	(284,723)	(248,333)
Long term borrowings	(194,706)	(195,466)	(195,466)	(195,466)	(195,466)
Other long term liabilities	(120,337)	(94,378)	(113,257)	(89,257)	(52,867)
Net Assets	14,856	(35,000)	(58,741)	(63,547)	(69,473)
CASH FLOW					
Operating Cash Flow	(67,780)	(75,003)	(1,125)	(38,087)	(20,727)
Net Interest	0	0	(5,375)	(5,375)	(5,375)
Tax	0	0	(26)	(26)	(26)
Capex	(1,009)	(394)	(2,772)	(2,466)	(2,855)
Acquisitions/disposals	0	0	0	0	0
Financing	(0)	0	0	0	0
Other	12,645	(51,021)	0	0	0
Dividends	0	0	0	0	0
Net Cash Flow	(56,143)	(126,418)	(9,298)	(45,955)	(28,984)
Opening net debt/(cash)	(226,125)	(169,982)	(43,564)	(34,266)	11,689
HP finance leases initiated	0	0	0	0	0
Other	0	0	0	0	0
Closing net debt/(cash)	(169,982)	(43,564)	(34,266)	11,689	40,673

Source: Edison Investment research, company reports

Contact details	Revenue by geography
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Management team

CEO: Mr Ronald Scott	CCO: Mr David Veitch
Mr Scott has been with Basilea since inception, initially as CFO until 2012 and then as COO until end 2012, and he has been CEO ever since. Before Basilea, Mr Scott spent nine years at Roche in senior positions in finance, licensing and M&A, and was involved in Roche's offerings of Genentech. Before entering the pharmaceutical industry, Mr Scott worked for Prudential Investment Corporation.	Mr Veitch joined Basilea in 2014 as chief commercial officer, having spent over 25 years in the pharmaceutical industry. Before Basilea, he was president of European operations at Savient Pharmaceuticals and spent 15 years at Bristol Myers Squibb, including leading the commercial operations in Europe, the Middle East and Asia. Mr Veitch holds a bachelor of science degree in biology.
CMO: Professor Achim Kaufhold	**CFO: Mr Donato Spota**
Professor Kaufhold joined Basilea in 2010 as chief medical officer, having spent 20 years in senior positions in the healthcare industry and 10 years as an academic. Before joining Basilea he was CEO of Affitech (Pharmexa) and held senior positions at Chiron (Novartis), Berna Biotech (Crucell/J&J) and GSK. He is a specialist in medical microbiology and infectious diseases.	Mr Spota joined Basilea in 2002 and became CFO in 2013. Before Basilea he worked for Roche. Mr Spota has more than 16 years of experience in the pharmaceutical industry, including finance, strategic financial planning and analysis, as well as audit and risk management. He holds an MBA from the University of Applied Sciences in Nürtingen, Germany.

Principal shareholders*	(%)
Franklin Resources Inc.	4.99%
CI Investments	5.07%
Credit Suisse	3.28%

* SIX Stock Exchange Regulation"

Companies named in this report

Actavis (ACT US); Astellas (4503 JP); Gilead (GILD US); Merck (MRK US); Pfizer (PFE US); Roche (ROG VX); Theravance (THRX US); Hikma

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