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bluebird bio

Pharma & biotech

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Poised to advance gene therapy pipeline

bluebird is advancing a pipeline focused on gene therapy, a rapidly expanding area that has attracted significant industry interest. It aims to offer one-time, transformative therapies for rare diseases, which should support premium pricing, if commercialised. Its two clinical programmes, LentiGlobin and Lenti-D, have been granted orphan drug designation in the US and Europe. A CAR-T oncology collaboration with industry leader Celgene validates the technology's promise.

Promising early clinical data for orphan programmes

LentiGlobin is in Phase I/II studies for blood disorders beta-thalassemia (15,000 US/Europe patients) and sickle cell disease (SCD, 20-25m global cases). In June 2014, interim data showed two beta-thalassemia patients remained transfusion-free for at least 3.5 months after treatment. A Phase I study for severe SCD is planned. Lenti-D is in Phase II/III for rare disorder childhood cerebral adrenoleukodystrophy (CCALD) and has shown stabilised neurological function, with no adverse events.

Celgene collaboration validates technology platform

bluebird's technology platform includes production of lentiviral vectors, which are used to transduce isolated target cells ex-vivo; those cells are subsequently infused back into the patient. This approach offers enhanced purity, potency and scalability over older vector gene therapies, and has shown promising safety to date. In 2013, Celgene entered into an oncology collaboration with bluebird to develop cancer products by applying gene therapy to modify a patient's T-cells. Celgene holds licence options on identified targets on Phase I completion.

Strong capital position supports strategic moves

bluebird had a pro-forma Q214 cash balance of $286m, after raising $110m net from a 3.45m share offering at $34 in July 2014. In June 2014, bluebird strengthened its technology platform by acquiring privately-held gene editing technology developer Pregenen for $20m in stock/assumed liabilities.

Valuation: Pipeline maturation should provide upside

bluebird's current EV of c $840m reflects positive sentiment and expectations for its pipeline, for which it holds worldwide rights, although it is constrained somewhat by the early stage of development. Additional data and pipeline advancement could unlock significant value, if efficacy and safety are confirmed. If a one-time, curative therapy, with orphan drug status, is commercialised, premium pricing is likely.

Consensus estimates

Year end	Revenue ($m)	PBT ($m)	EPS ($)	DPS ($)	P/E (x)	Yield (%)
12/12	0.34	(23.67)	(13.79)	N/A	N/A	N/A
12/13	20.18	(25.32)	(2.02)	N/A	N/A	N/A
12/14e	24.28	(49.03)	(1.74)	N/A	N/A	N/A
12/15e	24.90	(53.30)	(1.88)	N/A	N/A	N/A

Source: Company accounts and Bloomberg

Price	$40
Market cap	$1.14bn

Share price graph

Share details

Code	BLUE
Listing	NASDAQ
Shares in issue	28.6m

Business description

bluebird bio is a clinical-stage biotechnology company focused on using gene therapy to transform the lives of patients with severe genetic and orphan diseases. It offers the potential for one-time, curative treatments, which would support premium pricing for its rare disease candidates.

Bull

- One-time therapies for orphan-designated conditions would support premium pricing.
- Well funded to support current operations into 2017.
- Celgene CAR-T oncology collaboration validates technology and pipeline prospects.

Bear

- Safety needs to be confirmed in larger studies.
- Pipeline is not expected to yield a marketed product for several years.
- Rapidly evolving landscape could bring competition in target markets.

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