today's video we'll be talking about
beam therapeutics ticker symbol b-e-a-m
and comparing them to crispr
therapeutics ticker symbol c-r-s-p
they are both companies in the business
of designing gene editing systems
to correct underlying genetic disease
we'll talk about their technology
how it's similar and how it's different
we'll talk about the ceo
of beam we'll talk about their pipeline
and
much more on this channel we talk about
companies in the genomic space
healthcare sector biotech and much more
we've been talking about bio
nanogenomics bingo since they were
trading around two dollars talking about
sensionics
when they were one dollar a census when
they were one dollar
as well we've also talked about a number
of other gene editing companies
this is the place to be if you like to
learn more about the science of these
stocks
before they potentially take off so
please like
subscribe and consider joining our
patreon which we recently started
[Music]
beam therapeutics a new class of
precision genetic
medicines beam is pioneering the use of
base editing
a potential new class of precision
genetic medicines with a vision
of providing lifelong cures to patients
suffering from serious diseases
the dna is the underlying blueprint that
codes for the proteins which are the
functional units of
the body and they perform the tasks that
are required
to live and function however if you have
an abnormal
dna blueprint then you will create an
abnormal protein
and therefore have some type of genetic
disease
currently there are no cures for many
genetic diseases
because we haven't had a way to actually
correct the underlying blueprint

or the dna we've been waiting till the proteins are created
they perform their abnormal function and then we try and target
those proteins as best we can with various therapeutics
in order to understand how a base editor works which is what beam therapeutics has created
compared to crispr cast 9's system of gene editing you have to have a basic understanding of what dna
is and the different parts associated with it
dna is actually your blueprint and it is created
out of four letters the first of which is a the second is c
g and t as you can see here on the left
the letter t will always pair with a and g will always pair with c
and if you move from left to right you can see a sequence of different
letters and those letters are instructions for the creation of a given protein
number of diseases are caused by an abnormal letter within a sequence so if you substitute an
a for a c it will change the word
for example if you had the word the boy jumps if you remove
one of the letters for example o in boy and you replace it with a y
the sentence will not make much sense
and so if you remove
a t and you replace it with a c you're going to get an
abnormally shapen protein in some instances
this is where beam therapeutics technology comes
in they describe it as a pencil with an eraser for example if you're at this dna sequence
where you have the letter t if this t is incorrect
they can actually go in with their base editor
remove just the t and correct it to
a c for example and then they would be able to also correct the letter a
and replace it with a g without disrupting the rest of the dna sequence

now if we compare beam therapeutics base editor system
which is like a pencil and eraser where you can just erase the letter t
and replace it with the correct letter
you can't do that with the crispr cast 9 system which crispr therapeutics uses
for their gene editing system they actually use what's known as a barcode and scissors method
so rather than being able to snip out a single letter for example the letter
a you actually have to cut out a segment of a number of nucleotides so you might have to cut out c
a t a t g all in one
cut that segment out using the scissors of the crisper cast 9 system and then replace it with new
dna the problem that occurs when you're using the crisper cast 9
barcode scissor system is that when you cut out
larger segments of these nucleotides or letters
when the dna goes back to repair itself
it has the potential to introduce errors or add
incorrect letters to the mix and that can disrupt the whole sequence
and that may not be a problem if you're trying to knock out an abnormal
gene then that wouldn't be a problem but if you're just trying to correct a
single letter it would be more efficient and appropriate to potentially use the beam system
to correct that single nucleotide
because it avoids the risk
of introducing insertions or deletions that are
incorrect to get a better understanding of what i mean let's actually take a
look at how the crispr cast 9 system uses their barcode scissor system to cut at the dna
the crispr cas9 gene editing process begins when the complex recognizes and
binds to a short segment of dna
adjacent to the target site this initiates
unwinding of the dna helix which allows the guide rna to pair with a specific

target sequence in the dna
so the crispr cast 9 system which crispr therapeutics uses is described in this video
the cast 9 is the scissors that cuts at this double stranded
helix or the double-stranded dna and it's directed to
a given location within the dna which is defective
by using the crispr system
if the sequences pair precisely cas9 cuts the dna
forming a double strand break our cells respond to such breaks by activating natural dna repair pathways
those double-stranded breaks can introduce errors into that genetic sequence in some cases a process called non-homologous end joining
results in the additional deletion of a few base pairs
which disrupts the original dna sequence and can cause gene inactivation
a larger fragment of dna can also be removed by using
two different guide rnas to target separate sites on either side of the desired deletion
cleavage occurs at each site and the repair process joins the separate ends thereby deleting the intervening sequence
[Applause]
correction so as you can see in order to remove
or delete certain areas of the genome you actually have to cut out larger segments and be a little bit more creative using the crispr cast 9 system compare that with beams base editing system you can actually
cut out a single nucleotide at a time and so it's more efficient and less error prone
to dna can also be made by adding a dna template
along with a cas9 guide rna complex
the template is designed with sequences that exactly match the dna adjacent to the target cut
site through a process called homology directed repair
the cell uses the template to repair the

break thereby replacing the faulty dna sequence
or even inserting a new gene
that's how crispr cast 9 system uses their technology to replace defective dna they have to bring in a homologous sequence of dna to actually fit into the area that's been
cut out using their barcode scissor system as they describe it
let's take a look at the advantages to base editing compared to crispr cast 9 which we just
looked at first of which the creation of precise
predictable and efficient genetic outcomes at a targeted sequence
number two high efficiency editing without need for template based homology directed repair
they don't have to bring in an extra sequence of
dna to correctly edit a segment that's been cut
is what they're describing here and three avoidance of the unwanted consequence of double stranded dna breaks so anytime you cut dna
as we saw in that crispr cast 9 video there is the possibility
of inserting incorrect fragments of dna such as frequent insertions and deletions or larger scale genomic rearrangements should mention
that after listening to the ceo dr cole carney
from crispr therapeutics they have stated that their technology is very safe
and they haven't had any issues with off target
cuts before we move on we should talk about dr david liu phd who
is the founder of this technology as well as the company beam therapeutics and editos he is a richard merkin professor and vice chair of faculty at the broad institute of harvard and mit professor of chemistry and chemical biology at harvard university
and howard hughes medical institute investigator
he has published more than 150 papers and is an inventor of more than 50

issued patents i took a look a lot of
his publications
are in nature which is one of the most
prestigious journals
in all of scientific literature his work
on base editing has spurred numerous
publications within the healthcare
field publications have already come out
describing the base editing system
to be effective in treating diseases in
animal models for dr
lou is actually quite prolific within
the field of gene editing
and some have said that base editing is
an evolution
from crispr cast 9 and may be a better
more efficient system as we have
described taking a look at their
pipeline they are working towards
curative
disease pathways for sickle cell anemia
beta thalassemia
as well as looking in immuno-oncology
disease such as t cell acute
lymphoblastic leukemia
they're also trying to treat rare
diseases alpha-1 anti-trypsin deficiency
glycogen storage disorder 1a and are
also looking
into the treatment of ocular disease
ocular disease has been the focus of the
company editos
and hematologic conditions have been the
focus of crispr therapeutics
as well as intelia beam therapeutics is
much earlier
in their clinical work than crispr
therapeutics crispr therapeutics
has already conducted phase 1 phase ii
clinical work
in the treatment of sickle cell anemias
actually published their work
in new england journal of medicine
whereas this company is trying to treat
sickle cell
disease using their base editing system
but they are currently only
in the ind enabling period that is when
you submit an application
to the fda to gain approval to actually
begin clinical work
again this company was founded in 2017
compared to crispr therapeutics which
was founded in 2013

currently beam therapeutics is trading
at about half the market cap
of crispr therapeutics crispr
therapeutics they've already published
data
regarding sickle cell anemia as well as
beta thalassemia
and those results have been quite
positive also dr cole carney
has described their work in
immuno-oncology and they believe that
they haven't had issues with off-target
cuts
or some of the issues that could arise
from their barcode scissor system
compared to the base editing system by
beam therapeutics
it appears that beam therapeutics base
editing system could be an evolution
and improvement on the current barcode
scissor system
that crispr therapeutics intelia and
editos use
however as you can see from their
pipeline they are much
earlier i have a position in both beam
therapeutics as well as
crispr therapeutics this is not medical
or financial advice
please do your own due diligence and
happy investing
[Music]