WEBVTT

NOTE duration: ”00:04:17.237”

NOTE Confidence: 0.9504513

00:00:04.559 --> 00:00:06.080 Gene is a piece of

NOTE Confidence: 0.9504513

00:00:06.080 --> 00:00:08.180 the DNA we call blueprint

NOTE Confidence: 0.9504513

00:00:08.320 --> 00:00:08.980 for life.

NOTE Confidence: 0.80820405

00:00:09.599 --> 00:00:11.700 DNA or gene give instruction

NOTE Confidence: 0.9212942

00:00:12.505 --> 00:00:13.885 that goes to the mRNA,

NOTE Confidence: 0.9032632

00:00:14.185 --> 00:00:15.005 then mRNA

NOTE Confidence: 0.9544159

00:00:15.385 --> 00:00:16.765 translate to the protein.

NOTE Confidence: 0.90890723

00:00:17.704 --> 00:00:19.485 The protein pretty much responsible

NOTE Confidence: 0.90890723

00:00:19.625 --> 00:00:21.244 for all our biological

NOTE Confidence: 0.8801931

00:00:22.025 --> 00:00:22.525 functions.

NOTE Confidence: 0.7199942

00:00:22.985 --> 00:00:24.425 That's in how we work

NOTE Confidence: 0.7199942

00:00:24.425 --> 00:00:25.485 because our protein.

NOTE Confidence: 0.9076063

00:00:26.410 --> 00:00:27.770 Our gene determines which protein

NOTE Confidence: 0.9076063

00:00:27.770 --> 00:00:28.590 to be expressed.

NOTE Confidence: 0.91193247

00:00:29.690 --> 00:00:31.450 Every cell you have to

NOTE Confidence: 0.91193247

00:00:31.450 --> 00:00:31.950 have

NOTE Confidence: 0.8097397

00:00:32.409 --> 00:00:33.310 a sort of DNA

NOTE Confidence: 0.9016387

00:00:33.690 --> 00:00:35.470 to make the cell functions.

NOTE Confidence: 0.9016387

00:00:35.530 --> 00:00:37.070 But cells in different organs,

NOTE Confidence: 0.9016387

00:00:37.130 --> 00:00:38.590 different tissues, they

NOTE Confidence: 0.8639436

00:00:39.395 --> 00:00:41.254 express certain subset of genes.

NOTE Confidence: 0.8593061

00:00:44.034 --> 00:00:45.555 The genetic variant can cause

NOTE Confidence: 0.8593061

00:00:45.555 --> 00:00:47.094 called a personal trait,

NOTE Confidence: 0.90129477

00:00:47.555 --> 00:00:49.175 which is neutral benign.

NOTE Confidence: 0.95837086

00:00:49.635 --> 00:00:50.594 But some of them are

NOTE Confidence: 0.95837086

00:00:50.594 --> 00:00:52.754 crucial for our health. If

NOTE Confidence: 0.95837086

00:00:52.754 --> 00:00:54.295 that particular variant

NOTE Confidence: 0.904785

00:00:54.780 --> 00:00:55.740 end up in a very

NOTE Confidence: 0.904785

00:00:55.740 --> 00:00:57.600 critical part of the gene

NOTE Confidence: 0.920289

00:00:58.380 --> 00:01:00.220 We may produce a faulty

NOTE Confidence: 0.920289

00:01:00.220 --> 00:01:00.720 protein

NOTE Confidence: 0.84698343

00:01:01.260 --> 00:01:02.560 or, you know, we cannot

NOTE Confidence: 0.9835928

00:01:02.940 --> 00:01:04.620 produce a specific protein at

NOTE Confidence: 0.9835928

00:01:04.620 --> 00:01:05.120 all.

NOTE Confidence: 0.8572615

00:01:05.500 --> 00:01:07.920 That became disease causing. Oftentimes,

NOTE Confidence: 0.68411875

00:01:08.645 --> 00:01:09.385 we commutation.

NOTE Confidence: 0.8960001

00:01:09.925 --> 00:01:11.605 For some genetic disease, you

NOTE Confidence: 0.8960001

00:01:11.605 --> 00:01:11.765 know,

NOTE Confidence: 0.9650582

00:01:12.485 --> 00:01:13.685 the cause could be very

NOTE Confidence: 0.9650582

00:01:13.685 --> 00:01:15.785 complicated. Without understanding the mechanism,

NOTE Confidence: 0.9650582

00:01:15.925 --> 00:01:17.765 it's certainly very difficult to

NOTE Confidence: 0.9650582

00:01:17.765 --> 00:01:19.225 design any, treatment.

NOTE Confidence: 0.84420854

00:01:22.770 --> 00:01:25.250 Gene editing can modulate or

NOTE Confidence: 0.84420854

00:01:25.250 --> 00:01:26.390 correct a gene,

NOTE Confidence: 0.98773813

00:01:26.690 --> 00:01:28.049 which you believe in causing

NOTE Confidence: 0.98773813

00:01:28.049 --> 00:01:28.549 disease.

NOTE Confidence: 0.952841

00:01:29.170 --> 00:01:30.530 It's a very powerful tool

NOTE Confidence: 0.952841

00:01:30.530 --> 00:01:31.810 which could be used to

NOTE Confidence: 0.952841

00:01:31.810 --> 00:01:32.869 produce the

NOTE Confidence: 0.8724197

00:01:33.170 --> 00:01:34.549 the right form of protein,

NOTE Confidence: 0.8441189

00:01:35.009 --> 00:01:37.270 which respond for most, biological

NOTE Confidence: 0.99792606

00:01:37.569 --> 00:01:38.069 functions.

NOTE Confidence: 0.8222499

00:01:39.245 --> 00:01:41.584 Before, there is no tool

NOTE Confidence: 0.9849315

00:01:42.125 --> 00:01:43.485 can do this job very,

NOTE Confidence: 0.9849315

00:01:43.485 --> 00:01:44.305 very efficiently.

NOTE Confidence: 0.82092476

00:01:45.165 --> 00:01:46.845 Even somebody carries a genetic

NOTE Confidence: 0.82092476

00:01:46.845 --> 00:01:47.345 variance,

NOTE Confidence: 0.972304

00:01:47.805 --> 00:01:49.085 this genetic variance may not

NOTE Confidence: 0.972304

00:01:49.085 --> 00:01:51.505 affect all cells or tissues.

NOTE Confidence: 0.9391453

00:01:52.400 --> 00:01:54.640 When CRISPR coming, so this

NOTE Confidence: 0.9391453

00:01:54.640 --> 00:01:55.940 became a reality.

NOTE Confidence: 0.94400233

00:01:56.560 --> 00:01:58.400 So you can editing or

NOTE Confidence: 0.94400233

00:01:58.400 --> 00:02:00.320 correct the gene very efficient

NOTE Confidence: 0.94400233

00:02:00.320 --> 00:02:00.820 way.

NOTE Confidence: 0.78433645

00:02:01.120 --> 00:02:02.640 Basically, you can target at

NOTE Confidence: 0.78433645

00:02:02.640 --> 00:02:03.940 the specific cells.

NOTE Confidence: 0.82591087

00:02:04.255 --> 00:02:05.135 So there's a lot of

NOTE Confidence: 0.82591087

00:02:05.135 --> 00:02:06.995 advantages that makes CRISPR,

NOTE Confidence: 0.92925805

00:02:07.775 --> 00:02:08.595 so revolutionary.

NOTE Confidence: 0.8920011

00:02:09.375 --> 00:02:10.415 And then, you know, now

NOTE Confidence: 0.8920011

00:02:10.415 --> 00:02:12.495 a lot of, people, including

NOTE Confidence: 0.8920011

00:02:12.495 --> 00:02:12.995 us,

NOTE Confidence: 0.9438109

00:02:13.455 --> 00:02:14.755 try to use this technology

NOTE Confidence: 0.9438109

00:02:14.895 --> 00:02:15.855 to, you know, to treat

NOTE Confidence: 0.9438109

00:02:15.855 --> 00:02:16.755 genetic disease.

NOTE Confidence: 0.7921138

00:02:21.959 --> 00:02:23.639 Pretty much the biological structure

NOTE Confidence: 0.7921138

00:02:23.639 --> 00:02:24.840 of the brain makes drug

NOTE Confidence: 0.7921138

00:02:24.840 --> 00:02:25.959 delivery to the brain. This

NOTE Confidence: 0.7921138

00:02:25.959 --> 00:02:27.639 case deliver genome medicines to

NOTE Confidence: 0.7921138

00:02:27.639 --> 00:02:29.500 the brain. Very, very challenging.

NOTE Confidence: 0.86186314

00:02:30.595 --> 00:02:32.215 This very innovative technology,

NOTE Confidence: 0.7629757

00:02:32.514 --> 00:02:34.055 in short we call STAP,

NOTE Confidence: 0.7629757

00:02:34.114 --> 00:02:34.614 allow

NOTE Confidence: 0.99810535

00:02:35.075 --> 00:02:36.215 us to deliver

NOTE Confidence: 0.92553526

00:02:36.834 --> 00:02:37.974 the crisp editing

NOTE Confidence: 0.98568267

00:02:38.435 --> 00:02:39.255 more efficient

NOTE Confidence: 0.9941972

00:02:39.635 --> 00:02:40.455 to the brain.

NOTE Confidence: 0.89885974

00:02:41.635 --> 00:02:43.440 Basically, it's a technology based

NOTE Confidence: 0.89885974

00:02:43.440 --> 00:02:44.639 on the use of small

NOTE Confidence: 0.89885974

00:02:44.639 --> 00:02:46.720 molecules to deliver protein based

NOTE Confidence: 0.89885974

00:02:46.720 --> 00:02:48.340 genome editing machinery.

NOTE Confidence: 0.7552523

00:02:50.160 --> 00:02:52.100 We have developed a thousand

NOTE Confidence: 0.7552523

00:02:52.160 --> 00:02:53.780 of stuff molecules already.

NOTE Confidence: 0.90516776

00:02:54.240 --> 00:02:55.840 So we screen those stuff

NOTE Confidence: 0.90516776

00:02:55.840 --> 00:02:56.340 molecules.

NOTE Confidence: 0.91059303

00:02:57.025 --> 00:02:58.785 We found certain stuff molecules

NOTE Confidence: 0.91059303

00:02:58.785 --> 00:03:00.565 like a specific cell type.

NOTE Confidence: 0.7827858

00:03:01.985 --> 00:03:02.805 They enable,

NOTE Confidence: 0.92151797

00:03:03.185 --> 00:03:05.125 deliver all the CRISPR machinery

NOTE Confidence: 0.8336816

00:03:05.825 --> 00:03:07.665 into the brain and dispose

NOTE Confidence: 0.8336816

00:03:07.665 --> 00:03:09.264 the swabs brain. So that's

NOTE Confidence: 0.8336816

00:03:09.264 --> 00:03:10.005 very unique.

NOTE Confidence: 0.9214355

00:03:11.510 --> 00:03:13.030 We have five thousand disease.

NOTE Confidence: 0.9214355

00:03:13.030 --> 00:03:14.090 We know the mechanism,

NOTE Confidence: 0.9991709

00:03:14.469 --> 00:03:14.969 but

NOTE Confidence: 0.956084

00:03:15.430 --> 00:03:16.469 each one may have a

NOTE Confidence: 0.956084

00:03:16.469 --> 00:03:18.409 different mechanism. So therefore,

NOTE Confidence: 0.9191793

00:03:19.030 --> 00:03:20.730 individual disease, you may have

NOTE Confidence: 0.5382396

00:03:21.750 --> 00:03:22.889 developed individual

NOTE Confidence: 0.89569104

00:03:23.269 --> 00:03:24.230 sort of for the tool,

NOTE Confidence: 0.89569104

00:03:24.230 --> 00:03:25.750 even under the umbrella for

NOTE Confidence: 0.89569104

00:03:25.750 --> 00:03:26.915 the CRISPR editing.

NOTE Confidence: 0.87064725

00:03:28.095 --> 00:03:29.215 So far, I think we

NOTE Confidence: 0.87064725

00:03:29.215 --> 00:03:30.114 used the Cas9

NOTE Confidence: 0.88706493

00:03:30.575 --> 00:03:32.415 for the disease we're working

NOTE Confidence: 0.88706493

00:03:32.415 --> 00:03:33.855 on right now. It's Angelman

NOTE Confidence: 0.88706493

00:03:33.855 --> 00:03:35.795 syndrome. That potentially can

NOTE Confidence: 0.9770776

00:03:36.255 --> 00:03:37.775 apply to a few hundred

NOTE Confidence: 0.9770776

00:03:37.775 --> 00:03:39.635 other new genetic disease.

NOTE Confidence: 0.9114346

00:03:41.370 --> 00:03:42.490 Our hope is that in

NOTE Confidence: 0.9114346

00:03:42.490 --> 00:03:43.690 the next few years with

NOTE Confidence: 0.9114346

00:03:43.690 --> 00:03:45.310 the support from NIOSH,

NOTE Confidence: 0.9125769

00:03:45.770 --> 00:03:47.290 we can move this stuff

NOTE Confidence: 0.9125769

00:03:47.290 --> 00:03:48.990 based therapy into clinic.

NOTE Confidence: 0.81923926

00:03:50.730 --> 00:03:52.250 But stuff technology is a

NOTE Confidence: 0.81923926

00:03:52.250 --> 00:03:53.310 platform technology,

NOTE Confidence: 0.9629445

00:03:54.245 --> 00:03:55.525 which means that we can,

NOTE Confidence: 0.85509324

00:03:56.005 --> 00:03:57.864 potentially adapt this technology,

NOTE Confidence: 0.84450567

00:03:58.405 --> 00:04:00.584 for deliver our other, payloads

NOTE Confidence: 0.84450567

00:04:00.645 --> 00:04:02.345 to other cells and tissues.

NOTE Confidence: 0.8372529

00:04:04.724 --> 00:04:05.925 That means in long term,

NOTE Confidence: 0.8372529

00:04:05.925 --> 00:04:07.545 we hope, this technology

NOTE Confidence: 0.9029423

00:04:08.062 --> 00:04:09.502 can be repurposed enough for

NOTE Confidence: 0.9029423

00:04:09.502 --> 00:04:11.182 treatment of many other rare

NOTE Confidence: 0.9029423

00:04:11.182 --> 00:04:11.682 disease.