GENE THERAPY

Non-Discrimination Statement and Multi-Language Interpreter Services information are located at the end of this document.

Coverage for services, procedures, medical devices and drugs are dependent upon benefit eligibility as outlined in the member's specific benefit plan. This Medical Coverage Guideline must be read in its entirety to determine coverage eligibility, if any.

This Medical Coverage Guideline provides information related to coverage determinations only and does not imply that a service or treatment is clinically appropriate or inappropriate. The provider and the member are responsible for all decisions regarding the appropriateness of care. Providers should provide BCBSAZ complete medical rationale when requesting any exceptions to these guidelines.

The section identified as “Description” defines or describes a service, procedure, medical device or drug and is in no way intended as a statement of medical necessity and/or coverage.

The section identified as “Criteria” defines criteria to determine whether a service, procedure, medical device or drug is considered medically necessary or experimental or investigational.

State or federal mandates, e.g., FEP program, may dictate that any drug, device or biological product approved by the U.S. Food and Drug Administration (FDA) may not be considered experimental or investigational and thus the drug, device or biological product may be assessed only on the basis of medical necessity.

Medical Coverage Guidelines are subject to change as new information becomes available.

For purposes of this Medical Coverage Guideline, the terms "experimental" and "investigational" are considered to be interchangeable.

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GENE THERAPY (cont.)

Description:

Gene therapy has been investigated as a method to correct a defective gene, or to prevent or treat disease. Approaches include:

- Inserting a normal gene to replace an abnormal gene (most common approach)
- Exchanging an abnormal gene for a normal gene
- Mending an abnormal gene
- Changing the degree to which a gene is turned on or off

Criteria:

For chimeric antigen receptor T (CAR T) cell immunotherapy with Kymriah™ (tisagenlecleucel) criteria, refer to BCBSAZ Medical Coverage Guideline #O1037, “Kymriah™ (tisagenlecleucel)”.

For adoptive immunotherapy and chimeric antigen receptor T (CAR T) cell immunotherapy other than with Kymriah™ (tisagenlecleucel) criteria, refer to BCBSAZ Medical Coverage Guideline #O368, “Immunotherapy, Adoptive”.

- Gene therapy is considered experimental or investigational based upon:
  1. Insufficient scientific evidence to permit conclusions concerning the effect on health outcomes, and
  2. Insufficient evidence to support improvement of the net health outcome, and
  3. Insufficient evidence to support improvement of the net health outcome as much as, or more than, established alternatives, and
  4. Insufficient evidence to support improvement outside the investigational setting.

Resources:

Literature reviewed 01/30/18. We do not include marketing materials, poster boards and non-published literature in our review.


GENE THERAPY (cont.)

Resources: (cont.)


GENE THERAPY (cont.)

Resources: (cont.)


25. UpToDate.com. Chronic Granulomatous Disease: Treatment and Prognosis. 07/14/2015, 12/01/2013.


GENE THERAPY (cont.)

Non-Discrimination Statement:

Blue Cross Blue Shield of Arizona (BCBSAZ) complies with applicable Federal civil rights laws and does not discriminate on the basis of race, color, national origin, age, disability or sex. BCBSAZ provides appropriate free aids and services, such as qualified interpreters and written information in other formats, to people with disabilities to communicate effectively with us. BCBSAZ also provides free language services to people whose primary language is not English, such as qualified interpreters and information written in other languages. If you need these services, call (602) 864-4884 for Spanish and (877) 475-4799 for all other languages and other aids and services.

If you believe that BCBSAZ has failed to provide these services or discriminated in another way on the basis of race, color, national origin, age, disability or sex, you can file a grievance with: BCBSAZ’s Civil Rights Coordinator, Attn: Civil Rights Coordinator, Blue Cross Blue Shield of Arizona, P.O. Box 13466, Phoenix, AZ 85002-3466, (602) 864-2288, TTY/TDD (602) 864-4823, crc@azblue.com. You can file a grievance in person or by mail or email. If you need help filing a grievance BCBSAZ’s Civil Rights Coordinator is available to help you. You can also file a civil rights complaint with the U.S. Department of Health and Human Services, Office for Civil Rights electronically through the Office for Civil Rights Complaint Portal, available at https://ocrportal.hhs.gov/ocr/portal/lobby.jsf, or by mail or phone at: U.S. Department of Health and Human Services, 200 Independence Avenue SW., Room 509F, HHH Building, Washington, DC 20201, 1-800-368-1019, 800-537-7697 (TDD). Complaint forms are available at http://www.hhs.gov/ocr/office/file/index.html

Multi-Language Interpreter Services:

Spanish: Si usted, o alguien a quien usted está ayudando, tiene preguntas acerca de Blue Cross Blue Shield of Arizona, tiene derecho a obtener ayuda e información en su idioma sin costo alguno. Para hablar con un intérprete, llame al 602-864-4884.

Navajo: Díí kwe’ é atah nilínígíí Blue Cross Blue Shield of Arizona haada yit’éego bina’iidiłkidgo éí doodago Háida bijníi aniyeedigíí t’áadoo le’e yina’idlkidgo beehaz’áanii hólo díí t’áá hazaak’ehií háká a’dooowolgo bee haz’a doo báą́h ilínígóó. Ata’ halne’igíí kojí bich’éjí hodihíihá 877-475-4799.

Chinese: 如果您，或是您正在協助的對象，有關於插入項目的名稱 Blue Cross Blue Shield of Arizona 方面的問題，您有權利免費以您的母語得到幫助和訊息。洽詢一位翻譯員，請撥電話 在此插入數字 877-475-4799。

Vietnamese: Nếu quý vị, hay người mà quý vị đang giúp đỡ, có câu hỏi về Blue Cross Blue Shield of Arizona quý vị sẽ có quyền được giúp và có thể thông tin bằng ngôn ngữ của mình miễn phí. Để nói chuyện với một thợ dịch viên, xin gọi 877-475-4799.

Arabic: إن كان لديك أو لدى شريك تسامحك أسلحة بخصوص قضايا الضرورية بلغيتك من دون إعاقة للفقرة، للتحدث مع مترجم الصل ب 877-475-4799.
GENE THERAPY (cont.)

Multi-Language Interpreter Services: (cont.)

Tagalog: Kung ikaw, o ang iyong tinutulungan, ay may mga katanungan tungkol sa Blue Cross Blue Shield of Arizona, may karapatan ka na makakuha ng tulong at impormasyon sa iyong wika ng walang gastos. Upang makausap ang isang tagsalog, tumawag sa 877-475-4799.

Korean: 만약 귀하 또는 귀하가 돕고 있는 어떤 사람이 Blue Cross Blue Shield of Arizona에 대해 문의하신다면 귀하의 언어로 이용 부담없이 응답할 수 있는 권리가 있습니다. 그렇기 때문에, 귀하가 877-475-4799로 전화해주시면 된다는 것을 알려드립니다.

French: Si vous, ou quelqu’un que vous êtes en train d’aider, a des questions à propos de Blue Cross Blue Shield of Arizona, vous avez le droit d’obtenir de l’aide et l’information dans votre langue à aucun coût. Pour parler à un interprète, appelez 877-475-4799.

German: Falls Sie oder jemand, dem Sie helfen, Fragen zum Blue Cross Blue Shield of Arizona haben, haben Sie das Recht, kostenlose Hilfe und Informationen in Ihrer Sprache zu erhalten. Um mit einem Dolmetscher zu sprechen, rufen Sie bitte die Nummer 877-475-4799 an.

Russian: Если у вас или лица, которому вы помогаете, имеются вопросы по поводу Blue Cross Blue Shield of Arizona, то вы имеете право на бесплатное получение помощи и информации на вашем языке. Для разговора с переводчиком позвоните по телефону 877-475-4799.

Japanese: ご本人様、またはお客様の身の回りの方でも、Blue Cross Blue Shield of Arizonaについてご質問がございましたら、ご希望の言語でサポートを受けたり、情報を入手したりすることができます。料金はかからないです。通訳をお話される場合、877-475-4799までお電話ください。

Farsi:

آگر شما، یا کسی که شما به آن کمک می‌کنید، سوالی می‌ورده، درخواست دریافت اطلاعات به زبان خود را به طور رایگان دریافت نمایید 877-475-4799.

Assyrian:

Blue Cross Blue Shield of Arizona ئەنەوەی، ئەگەر خۆکەیەکەیە، ئەگەر خۆکەیەکەیە، ئەگەر خۆکەیەکەیە، ئەگەر خۆکەیەکەیە، ئەگەر خۆکەیەکەیە، ئەگەر خۆکەیەکەیە، ئەگەر خۆکەیەکەیە، ئەگەر خۆکەیەکەیە، ئەگەر خۆکەیەکەیە، ئەگەر خۆکەیەکەیە، ئەگەر خۆکەیەکەیە.

Serbo-Croatian: Ukoiko Vi ili neko kom Vi pomažete ima pitanje o Blue Cross Blue Shield of Arizona, imate pravo da besplatno dobijate pomoć i informacije na Vašem jeziku. Da biste razgovarali sa prevodiocem, nazovite 877-475-4799.

Thai: หากคุณ หรือคนที่คุณช่วยเหลือต่างประเทศท่านอยู่ Blue Cross Blue Shield of Arizona คุณสามารถจะได้ความช่วยเหลือและข้อมูลภาษาของคุณได้โดยโทรศัพท์ที่ 877-475-4799.